Exhibit 10.15

Confidential Materials omitted and filed separately with the
Securities and Exchange Commission. Double asterisks denote omissions.

SPONSORED RESEARCH AGREEMENT

THIS SPONSORED RESEARCH AGREEMENT (the "***Agreement***") is entered into as of June 1, 2006 (the "***Effective Date***"), by and between **SPINAL MUSCULAR ATROPHY FOUNDATION** (the "***Foundation***"), having its principal place of business located at 1776 Broadway, 22nd Floor, New York, New York, 10019, and **PTC THERAPEUTICS, INC. (**"***PTC***" or "***Company***"), having its principal place of business located at 100 Corporate Court, South Plainfield, New Jersey, 07080.

RECITALS

WHEREAS, Company is focused on the discovery, development, and commercialization of small-molecule drugs targeting post-transcriptional control mechanisms;

WHEREAS, the Foundation is dedicated to accelerating the development of a treatment or cure for spinal muscular atrophy;

WHEREAS, the Foundation wishes to sponsor, and Company wishes to perform, research focused on small molecule therapeutics for spinal muscular atrophy ("***SMA***"), and possibly to further develop and commercialize such therapeutics, subject to the terms and conditions of this Agreement, including the Research Plan attached hereto as **Exhibit A**;

WHEREAS, it is the intent of the Foundation and Company to disseminate the results of the Research (as defined below) to other investigators in the spinal muscular atrophy research community and to medical professionals treating spinal muscular atrophy patients, consistent with the overall goal of commercializing therapeutics for SMA; and

WHEREAS, it is the further intent of the Foundation and Company that patents and other intellectual property developed by Company as a result of the Research shall be retained by Company, but that a mechanism be provided for transfer of rights to the patents and other intellectual property developed by Company as a result of the Research and relating to a particular Research Project (as defined below) to the Foundation if Company elects not to pursue commercial development of any drug candidates identified during such Research Project.

NOW, THEREFORE, in consideration of the foregoing and the mutual covenants and premises contained in this Agreement, the parties hereto agree as follows:

1. DEFINITIONS.

1.1 "Additional Payments" shall mean all amounts actually paid to Company pursuant to Section 4.2.

1.2 "Affiliate" shall mean any corporation or other entity that controls, is controlled by, or is under common control with, a party. A corporation or other entity shall be regarded as in control of another corporation or entity if it owns or directly or indirectly controls more than 50% of the voting securities or other ownership interest of the other corporation or entity, or if it possesses, directly or indirectly, the power to direct or cause the direction of the management and policies of the corporation or other entity.

1.3 "Available Product" shall mean a human therapeutic product that (a) has previously received final approval from the FDA for marketing in the United States and (b) is suitable for administration to patients in its currently marketed formulation for the treatment of spinal muscular atrophy.

1.4 "Company Base IP" shall mean any new and useful composition of matter, process, product by process, machine or manufacture, know-how, discovery, improvement, Patent, or other intellectual property ("**IP**") or any new and useful improvement thereof, whether or not patentable, which (i) is discovered, conceived, developed or first reduced to practice by or on behalf of Company as of or prior to the Effective Date, (ii) is an improvement to any IP discovered, conceived, developed or first reduced to practice by or on behalf of Company as of or prior to the Effective Date, regardless of when such improvement is discovered, conceived, developed or first reduced to practice, or (iii) is discovered, conceived, developed or first reduced to practice by, or otherwise comes under the Control of, Company during the Research Term and does not constitute Data or a Research Invention.

1.5 "Company Clinical Trial" shall have the meaning provided in Section 3.4.

1.6 "Company Know-How" shall mean Information that: (a) is developed or acquired by or on behalf of Company in the course of performing the Research; and/or (b) is otherwise Controlled by Company and is directed to any Drug Target, Hit, Lead Candidate, Drug Candidate or Product first identified or synthesized in the conduct of the Research, formulations of any of the foregoing, and/or processing technology with respect thereto; *provided, however,* that the Company Know-How excludes the Company Patents and the Company Base IP.

1.7 "Company Patents" shall mean Patents that: (a) claim Information developed or acquired by or on behalf of Company in the course of performing the Research; and/or (b) are otherwise Controlled by Company and claim any Drug Target, Hit, Lead Candidate, Drug Candidate or Product first identified or synthesized in the course of the Research, formulations of any of the foregoing, and/or processing technology with respect thereto; *provided, however,* that the Company Patents exclude the Company Base IP and the Company Know-How.

1.8 "Company Technology" shall mean Company Know-How and Company Patents.

1.9 "Confidential Information" shall mean any confidential or proprietary information of a party, including, without limitation, information relating to any compound, product specifications, chemical structures, data, know-how, formulations, research project, work in process, future development, scientific, engineering, manufacturing, marketing, business plan, financial or personnel matter relating to such party, its present or future products, sales, suppliers, customers, employees, investors or business, whether in oral, written, graphic or electronic form, subject to the provisions of Section 5.2 hereof. Without limiting the generality of the foregoing, the terms of this Agreement shall be deemed the Confidential Information of both parties, subject to Section 5.5.

1.10 "Control" shall mean, with respect to any Information, Patent or other intellectual property right, possession by a party of the ability (whether by ownership, license or

otherwise) to grant access, a license or a sublicense to such Information, Patent or other intellectual property right without (a) violating the rights of any Third Party or the terms of any agreement or other arrangement with any Third Party, and (b) incurring any additional cost or royalty obligation to such Third Party based on the granting of such access, license or sublicense.

1.11 "Data" shall have the meaning provided in Section 6.1(a).

1.12 "Drug Candidate" shall mean a Hit, Lead Candidate or any metabolite, prodrug, solvate (including without limitation any hydrate), ester, salt, stereoisomer, racemate, tautomer or polymorph of such Hit or Lead Candidate that is first synthesized or identified in the conduct of the Research and that exhibits desired levels of activity against the applicable Drug Target.

1.13 "Drug Target" shall mean a gene or other biological target described in the Research Plan or mutually agreed upon by both parties as having potential application for the identification and development of Drug Candidates for the prevention or treatment of SMA.

1.14 "FDA" shall mean the United States Food and Drug Administration (or its successor agency).

1.15 "Field" shall mean the treatment or prevention of [**].

1.16 "First Commercial Sale" shall mean the date of the first commercial sale in a country or region by or on behalf of Company or its Affiliate or Licensee of a Product to another party after Regulatory Approval has been obtained for such Product in such country or region.

1.17 "Hit" shall have the meaning provided in Section 2.4(a).

1.18 "IND" shall mean an Investigational New Drug Application filed with the FDA.

1.19 "Information" shall mean all tangible and intangible techniques, technology, practices, trade secrets, inventions (whether or not patentable), methods, knowledge, know-how, skill, experience, test data and results (including pharmacological, toxicological and clinical test data and results), analytical and quality control data, results or descriptions, software, algorithms, compositions of matter, cells, cell lines, assays, animal models and physical, biological or chemical material.

1.20 "IP Filing Period" shall have the meaning provided in Section 5.4.

1.21 "Lead Candidate" shall have the meaning provided in Section 2.4(a).

1.22 "Lead Optimization" shall mean shall mean a program of medicinal chemistry the intent of which is to develop a Lead Candidate into a compound or formulation suitable as the subject of an IND submission to the FDA.

1.23 "Licensee" shall mean a Third Party to whom Company or any of its Affiliates has granted a license or sublicense of the right to develop, make, have made, use, distribute for sale, promote, market, offer for sale, sell, have sold, import or export Drug Candidate or Product, beyond the mere right to purchase Drug Candidate or Product from Company or its Affiliates.

1.24 "Net Sales" shall mean the gross amounts received by Company and its Affiliates (but not their respective Licensees) following the First Commercial Sale of a Product for sales of such Product to Third Parties that are not Affiliates or Licensees of the selling party (unless such Affiliate or Licensee is the end user of such Product, in which case the amount billed therefor shall be deemed to be the amount that would be billed to a Third Party end user in an arm's-length transaction), less the following items, as allocable to such Product (if not previously deducted from the amount invoiced): (i) bad debts actually written off which are attributable to sales of Products; (ii) trade discounts, credits or allowances; (iii) credits, refunds or allowances additionally granted upon returns, rejections or recalls; (iv) freight, shipping and insurance charges; (v) taxes, duties or other governmental tariffs (other than income taxes); (vi) any payment in respect of sales to any governmental authority in respect of any government-subsidized program, including, without limitation, Medicare and Medicaid rebates; and (vii) distribution, packing, handling and transportation charges for Products to the extent that they are included in the price or otherwise paid by the customer.

1.25 "Patents" shall mean (a) United States and foreign patents, re-examinations, reissues, renewals, extensions and term restorations, and foreign counterparts thereof, and (b) pending applications for United States and foreign patents, including, without limitation, provisional applications, continuations, continuations-in-part, divisional and substitute applications, including, without limitation, inventors' certificates, and foreign counterparts thereof.

1.26 "Principal Scientist" shall mean Dr. Stuart Peltz.

1.27 "Product" shall mean a pharmaceutical product comprising or containing a Drug Candidate, including, in each case, all formulations, line extensions and modes of administration thereof.

1.28 "Product Revenues" shall mean Net Sales of Products by Company and its Affiliates, plus all royalties, license fees, milestone payments, annual maintenance fee or similar payment or consideration paid by a Licensee to Company or its Affiliates in consideration for the grant by Company or its Affiliate of a license to develop, make, have made, use, distribute for sale, promote, market, offer for sale, sell, have sold, import or export Drug Candidates or Products (with any of the foregoing consideration received by Company other than in the form of cash to be valued at its fair market value as of the date of receipt), minus any payments attributable to Product that are made by Company or its Affiliates in respect of a Third Party Patent License; *provided, however,* that "Product Revenues" shall in any event exclude any funds paid to directly support research and/or development actually being performed by Company or its Affiliates (in amounts that are commercially reasonable in light of the research and/or development services being performed), and payments for equity or debt securities of Company or its Affiliates (except to the extent such payments exceed the fair market value of such securities upon date of receipt, in which event such excess over fair market value shall be included in the calculation of Product Revenues).

1.29 "Regulatory Approval" shall mean any and all approvals (including pricing and reimbursement approvals), licenses, registrations or authorizations of any kind by the FDA or other applicable regulatory authority outside the U.S. necessary for the development, pre-clinical

and/or human clinical testing, manufacture, quality testing, supply, use, storage, importation, export, transport, pricing, marketing and/or sale of a Product for use in the Field.

1.30 "Research" shall mean the activities conducted pursuant to the Research Plan.

1.31 "Research Funds" shall mean all amounts actually paid to Company pursuant to Section 4.1.

1.32 "Research Invention" shall mean any new and useful composition of matter, process, product by process, machine or manufacture, know-how, discovery, improvement, or other intellectual property or any new and useful improvement

thereof, whether or not patentable, discovered, conceived, developed or first reduced to practice in the conduct of the Research.

1.33 "Research Milestone" shall have the meaning provided in Section 2.2.

1.34 "Research Plan" shall mean the research plan attached hereto as ***Exhibit A***, which is incorporated herein by this reference, as such research plan may be modified from time to time by mutual written agreement of the Foundation and Company.

1.35 "Research Project" shall mean any one of the constituent research projects that make up the Research, each identified by sequential lettering in the Research Plan.

1.36 "Research Term" shall have the meaning provided in Section 2.6.

1.37 "Research Tool" shall mean a Research Invention that may contribute to the identification or development of products useful in the Field, and that is none of the following: (a) Drug Candidate(s) identified by Company (or any of its corporate partners, Licensees, or sublicensees); (b) Product(s) based on or containing such Drug Candidate(s); or (c) Company Base IP. For the avoidance of doubt, the parties do not intend the definition of Research Tool to apply, in whole or in part, to any aspect of PTC's GEMS technology.

1.38 "Reversionary License" shall have the meaning provided in Section 6.1(c).

1.39 "SMA" shall mean spinal muscular atrophy.

1.40 "SMA Research Tools" shall mean any research tools of the Foundation or its Affiliates, or any Third Parties with which SMA has a relationship, which might be necessary or useful for the Research.

1.41 "Third Party" shall mean any entity other than the Foundation or Company or an Affiliate of the Foundation or Company.

1.42 "Third Party Patent License" shall have the meaning provided in Section 3.2.

2. CONDUCT OF THE RESEARCH.

2.1 Objective. Subject to the terms and conditions of this Agreement, the parties agree that, during the Research Term, Company shall perform the Research in accordance with the Research Plan, and each party shall contribute the materials and services specified therein, with the goal of identifying and developing small molecule therapeutics for use in the Field.

2.2 Research Plan; Contributions. The Research Plan sets forth the activities proposed to be conducted by Company, together with an anticipated schedule for completion of such activities. Company agrees to use commercially reasonable efforts to achieve the research milestones (the "***Research Milestones***") and research goal(s) described in Exhibit B (attached hereto) on the schedule set forth therein and to incorporate feedback from the Foundation's scientific advisors. The parties will jointly review the research goals, activities and schedule set forth in the Research Plan and may, by mutual written agreement, amend the Research Plan from time to time during the course of the Research Term and, in connection therewith, may (i) modify the funding amounts and schedule set forth in Section 4.1, (ii) add additional Research Milestones or goals to Exhibit B, or (iii) provide for Additional Payments, as appropriate. Each party shall contribute to the Research the materials and services specified in the Research Plan, and the Foundation shall use commercially reasonable efforts to assist Company in obtaining favorable licensing terms to SMA Research Tools necessary or useful for the Research.

2.3 Principal Scientist. The Principal Scientist is considered essential to the Research being performed, and no substitution may be made without the prior written agreement of the Foundation. If for any reason the Principal Scientist ceases to be employed by Company or otherwise becomes unavailable, or cannot continue to oversee the conduct or completion of the Research, Company will propose a successor whose appointment as Principal Scientist shall be subject to the approval of the Foundation, such approval not to be unreasonably withheld. If the parties are unable to agree upon a successor within 90 days after the Principal Scientist ceases his involvement in the Research, this Agreement may be terminated by the Foundation pursuant to Section 7.3.

2.4 The Research.

(a) During the Research Term, Company shall conduct each of the Research Projects in accordance with this Agreement and the Research Plan. Company shall disclose the results of all Research activities to the Foundation in accordance with Section 2.7. Company may select, after disclosing the applicable criteria to the Foundation, one or more compounds that have been validated in secondary assay(s) and have suitable *in vitro* potency, or otherwise meet the criteria set

forth in the Research Plan (or otherwise mutually agreed upon by the parties) for further evaluation (each such compound being hereinafter referred to as a ***"Hit"***), following which, as more fully described in the Research Plan, Company shall: (i) assess each Hit (and, as the parties deem appropriate consistent with the Research Plan, any analog, derivative or formulation thereof) with the goal of identifying one or more compounds that have suitable properties for administration to humans (each such compound being hereinafter referred to as a ***"Lead Candidate"***); and (ii) evaluate and, if appropriate based on such evaluation, optimize each Lead Candidate for therapeutic administration to humans.

(b) The parties shall mutually agree upon a strategy for medicinal chemistry follow-up on Lead Candidates and further pharmacology studies, formulation development, safety and toxicity studies, dosing studies or other preclinical work at Company, or at Company's option, through external collaboration or licensing with a Third Party. As promptly as practicable after identification of one or more Lead Candidates, Company shall provide the Foundation with total cost estimates for continued Lead Optimization and development of such Lead Candidates, and the Foundation may elect to fund, in the form of cash payments to Company, some, all or none of this work upon reasonable advance notice to Company. In addition, the Foundation may act to secure funding from Third Parties, and/or assist Company to obtain alternative sources of external funding, and in each case such funding would be administered through and governed by this Agreement as specified in a written agreement with any such Third Party, such Agreement to specify the impact of such alternative sources of funding on the payment obligations of the Company under Section 4.3.

(c) Company shall disclose the results of all Research activities regarding Hits and Lead Candidates to the Foundation in accordance with Section 2.7, and the parties shall consult with each other with the objective of identifying at least one Drug Candidate suitable for progression to the preparation and filing of an IND in the Field and, contingent on the effectiveness of such IND, progressing such Drug Candidate into human clinical trials in the most expeditious manner.

2.5 Performance Standards. Company shall conduct the Research in good scientific manner, and in compliance in all material respects with the requirements of applicable laws and regulations and with applicable good laboratory practices, to attempt to achieve its objectives efficiently and expeditiously. Company shall maintain (either as its own internal resources, or via subcontract) laboratories, offices and all other facilities reasonably necessary to carry out the activities to be performed by it pursuant to the Research Plan. In conformity with standard pharmaceutical and biotechnology industry practices and the terms and conditions of this Agreement, Company shall prepare and maintain, or shall cause to be prepared and maintained, complete and accurate written records, accounts, notes, reports and data with respect to activities conducted pursuant to the Research Plan. Upon reasonable advance notice, Company agrees to make its employees and non-employee consultants reasonably available at their respective places of employment to consult with the Foundation on issues or questions arising during the Research Term.

2.6 Research Term. The initial phase of the Research is expected to require one year to reach the primary overall objective of Lead Candidate identification. The parties may, from time to time, on a Research Project-by-Research Project basis, extend or modify the Research Term by mutual written agreement (the initial one-year period and any extensions or modifications together shall hereinafter referred to as the ***"Research Term"***).

2.7 Communication; Research Reports. On a regular basis during the Research Term (but no less frequently than [**]), the parties shall conduct meetings, either in person or by telephone or video conference, to discuss the progress of the Research and strategies for achieving the objectives of the Research in an expeditious manner. Company shall keep the Foundation fully informed as to all results and discoveries (including, without limitation, assay development and all Hits and potential Lead Candidates and Drug Candidates) made in the

course of performing activities under the Research Program at these meetings. In furtherance of the foregoing, on a [**] basis, Company shall prepare, and deliver to the Foundation no later than [**] days after the conclusion of [**] during the Research Term, a reasonably detailed written summary report of the results and progress of the Research during [**] (each, a ***"Research Report"***). In addition, the Foundation may, at its option, during the Research Term, schedule up to [**] formal program review meetings with Company personnel and those of Foundation's Third Party advisors who (i) have agreed to confidentiality restrictions substantially similar to those contained in this Agreement, and (ii) are reasonably acceptable to Company. Such meetings will be held at the times and locations mutually agreed upon by the parties. The purpose of such meetings will be to review the progress of the Research relative to the Research Plan.

2.8 Subcontracts. Company may perform some of its obligations under the Research Plan through one or more subcontractors, provided that (a) none of the rights of either party hereunder are diminished or otherwise adversely affected as a result of such subcontracting, and (b) Company will at all times be responsible for the performance and, except as otherwise agreed by the parties in writing, payment of such subcontractor; provided, however, that the Company may use payments received by it

pursuant to Section 4.1 to pay for such subcontractor(s). In determining whether any Company obligations under the Research Plan will be performed in-house or by a Third Party subcontractor, Company shall take into consideration Company's then-current capabilities and the relative efficiency of utilizing such internal capabilities versus Third Party services.

2.9 Additional Screening. The Foundation may request that Company test up to [**] compounds identified by other Foundation partners ***("Third Party Compounds"***) on a blinded basis. Company agrees to test such Third Party Compounds on behalf of the Foundation and to disclose the results of such screening to the Foundation, provided that the relevant assay is already being run by the Company on its own Compounds. Such testing shall be performed pursuant to a separate materials transfer agreement to be negotiated in good faith by the parties prior to provision of any compounds or related information, which agreement shall contain reasonable and customary terms to protect the parties' respective intellectual property rights. Without limiting the generality of the foregoing, each such materials transfer agreement shall provide that in no event shall any Third Party Compound become the property of the Company, nor shall any Third Party Compound become subject to royalty or other reach-through payment obligations to Company or its affiliates as a result of such screening by Company.

3. DEVELOPMENT OF PRODUCTS.

3.1 Clinical Development Strategy. As soon as Company reasonably believes that it has identified a Drug Candidate for which it proposes to file an IND in the Field, Company will notify the Foundation in writing, and the parties will promptly discuss in good faith how to proceed with the clinical development of such Drug Candidate, taking into consideration the interests of SMA patients, the intellectual property and regulatory landscape and the commercial potential of the Drug Candidate. The parties agree to consider in good faith collaborating with the NIH in preclinical or clinical development activities regarding such Drug Candidate. Should Company elect to proceed with clinical development of the Drug Candidate, it may do so directly. In the alternative, at its discretion, the Company may decide to enter into a

collaboration with one or more Third Parties for clinical development and/or commercialization of the Drug Candidate through licensing or other arrangement; *provided, however,* that if the Foundation has funded (or caused to be funded) [**], then until [**], any such collaborations shall be subject to the Foundation's approval (which shall not be unreasonably withheld). If Company wishes to pursue clinical development of a Drug Candidate, the Company will consult with the SMA Foundation on the clinical trial network that will be used. Although the parties currently expect to use the clinical trial network established by the Foundation, the clinical trial network to be used shall be determined in good faith by Company in its reasonable judgment. For any Drug Candidate for which it files an IND, Company agrees to consider in good faith whether to obtain, (a) "Orphan Product" designation from the FDA, and (b) research funding from the FDA's Office of Rare Diseases to support human clinical trials conducted for such Drug Candidate. The parties acknowledge that if the Drug Candidate is [**], or [**] due to [**] and [**], investment by Company in further development of such Drug Candidate may not be in the best interests of Company's stockholders, and therefore shall not be required under this Agreement, and the failure to engage in such further development shall not be the basis of a Reversionary License under Section 6.1(c). In such case, the parties may elect to enter into an additional sponsored research agreement under which the Foundation would provide funding for further development efforts by Company, but neither party shall have any obligation to enter into such additional agreement.

3.2 Conduct of Clinical Development. Except as set forth in Section 3.1 above or as otherwise agreed by the parties in writing, Company shall be responsible for clinical development of any Drug Candidate for which Company files an IND. Company shall use commercially reasonable efforts to develop and commercialize (whether directly, through an Affiliate, or in collaboration with one or more Third Parties, through licensing or some combination of the foregoing) at least one Product. The parties anticipate that an IND will be submitted within [**] years of commencement of IND-enabling toxicology studies for a Lead Candidate, but the parties acknowledge that [**], and therefore [**], to be a [**]. Notwithstanding the preceding provisions of this Section 3.2, in no event shall Company have any obligation (i) to pursue clinical development or commercialization of any Drug Candidate which is [**], or which [**] the [**] due to its [**] and [**], or (ii) in the absence of complete funding by (or arranged by) the Foundation, to pursue clinical development or commercialization of any Drug Candidate which is not, [**]. In addition, Company shall not be obligated to pursue clinical development or commercialization of a Drug Candidate if the pharmaceutical preparation, composition of matter, method of manufacture and/or method of use of such Drug Candidate is covered by Patents of a Third Party, unless a license under such Third Party Patents is available to Company (or its Affiliate or Licensee, as applicable) on commercially reasonable terms (a ***"Third Party Patent License"***).

3.3 Disclosure Regarding Company Efforts. Company will keep the Foundation appropriately informed about clinical trial progress and commercialization efforts with respect to Products, and in any event, Company shall provide the Foundation with [**] written reports summarizing any significant development or commercialization events that have occurred during the applicable [**]-month period, provided that such reports may be incorporated into any Research Reports then being prepared and delivered under Section 2.7.

4. PAYMENTS.

4.1 Research Funding by the Foundation. For the conduct of the Research, and subject to the completion of the applicable Research Milestones described in Exhibit B (attached hereto), the Foundation shall pay a total of US$[**] to Company on the schedule specified below:

(a) within [**] days after the Effective Date, the Foundation will pay to Company US$[**];

(b) within [**] days after the Foundation's receipt of notice from Company of the achievement of Milestone 1 in Exhibit B attached hereto, the Foundation will pay Company US$[**];

(c) within [**] days after the Foundation's receipt of notice from Company of the achievement of Milestone 2 in Exhibit B attached hereto, the Foundation will pay Company US$[**]; and

(d) within [**] days after the Foundation's receipt of notice from Company of the achievement of Milestone 3 in Exhibit B attached hereto, the Foundation will pay Company US$[**].

The Foundation may delay any payment until such time as the milestones in the Research Plan are met (or as may otherwise be mutually agreed in writing). For purposes of clarification, the foregoing payments shall be non-refundable, and each of the foregoing payments shall be payable only once. The Foundation acknowledges that the foregoing payments represent only a portion of the total cost of performing the Research. Notwithstanding the foregoing,, except as agreed pursuant to Section 4.2, the Foundation will not be obligated to pay any additional amounts in connection with the Research.

4.2 Additional Payments. In addition to the amounts specified in Section 4.1, upon mutual written agreement of the parties, the Foundation may make, or cause to be made, additional research funding payments to Company in connection with any modification of the Research Plan.

4.3 Milestone Donation by Company. Within [**] days after the end of the first fiscal quarter in which Company has received an aggregate of US$[**] in Product Revenues, Company shall make a payment to the Foundation (or, at the Foundation's option, one or more other non-profit organizations or academic or research institutions designated by the Foundation in writing) in the applicable amount set forth below pursuant to clause (a), (b) or (c), whichever **one** (and only one) of the following applies:

(a) [**];

(b) [**]; or

(c) [**].

In addition to the foregoing milestone payments, and provided that the Foundation provided funding for Lead Optimization of Products hereunder at the level set forth in the first paragraph of 4.3(c), within [**] days after the end of the first calendar year during which Company has received an annual aggregate in that year of US$[**] in Product Revenues, Company shall make a payment to the Foundation equal to 100% of the sum of the Research Funds and the Additional Payments. For the avoidance of doubt, such additional payment shall be a one-time payment only, regardless of any additional Product Revenues.

If [**] in good faith believes that making the applicable payment(s) specified in this Section 4.3 on the schedule set forth above will prevent Company from achieving a reasonable profit margin on commercial sales of Products, [**] may reduce any such payments due in the applicable calendar or fiscal quarter by [**]%, or such other reduction as the parties shall in good faith agree, with any reduction carried forward on a quarter-by-quarter basis (subject to the same reductions in each subsequent quarter) until paid in full.

4.4 Reporting of Product Revenues. From and after such time as Company first receives any Product Revenues and until such time as Company has paid in full the amount due under Section 4.3 (if any), Company shall deliver to the Foundation (or a Third Party designated in writing by the Foundation) quarterly written reports of Product Revenues received by Company and its Affiliates, which reports shall indicate the total Product Revenues received. Company shall keep, and shall cause its Affiliates to keep, complete and accurate records pertaining to the receipt of Product Revenues in sufficient detail to permit the Foundation to confirm the accuracy of such reports.

4.5 Exchange Rate; Manner and Place of Payment. All payments hereunder shall be payable in U.S. dollars. When conversion of payments from any foreign currency is required for purposes of calculating Product Revenues, such

conversion shall be at the exchange rate used by Company (or, where applicable, a Licensee) throughout its accounting system (which shall, in any event, be commercially reasonable) during the quarter for which such report is due. All payments owed under this Agreement shall be made by check, or by wire transfer in immediately available funds to a bank and account designated in writing by the party entitled to receive payment, unless otherwise specified in writing by such party.

4.6 Taxes. Each party will pay any and all taxes levied on account of any payments made to it under this Agreement out of the amounts it is to receive hereunder. If any taxes are required to be withheld by the party making payment, such party will (a) deduct such taxes from the payment made by it, (b) timely pay the taxes to the proper taxing authority, (c) send proof of payment to the other party and certify its receipt by the taxing authority within [**] days following such payment, and (d) be deemed to have paid such amount to the other party hereunder.

4.7 Audits. The Foundation shall have the right to cause an independent, certified public accountant reasonably acceptable to Company to audit the records of Company and its Affiliates to confirm the accuracy of Company's reports of Product Revenues for a period covering not more than the preceding [**] years. Such audits may be exercised during normal business hours upon reasonable prior written notice to Company and no more than [**] per year. Prompt adjustments shall be made by the parties to reflect the results of such audit. The

Foundation shall bear the full cost of such audit unless such audit discloses an underreporting of Product Revenues by Company of more than [**]% during any calendar year, in which case, Company shall bear the full cost of such audit.

5. CONFIDENTIALITY.

5.1 Confidentiality. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing by the parties, the parties agree that, during the Research Term and for a period of [**] years thereafter, each party (the ***"Receiving Party"***) will maintain in confidence all Confidential Information disclosed to it by the other party (the ***"Disclosing Party"***), provided that, with regard to Confidential Information which is trade secret information, such obligation shall extend thereafter until such information is no longer a trade secret of the Disclosing Party. The Receiving Party may use the Confidential Information of the Disclosing Party only to the extent required to accomplish the purposes of this Agreement. The Receiving Party shall use at least the same standard of care as it uses to protect proprietary or confidential information of its own to ensure that its employees, agents, consultants and other representatives do not disclose or make any unauthorized use of the Disclosing Party's Confidential Information. Each party will promptly notify the other upon discovery of any unauthorized use or disclosure of the other party's Confidential Information.

5.2 Exceptions. The obligations of confidentiality contained in Section 5.1 will not apply to the extent that it can be established by the Receiving Party by competent proof that such Confidential Information: (a) was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party; (b) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party; (c) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the Receiving Party in breach of this Agreement; (d) is independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or (e) was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others.

5.3 Authorized Disclosure. Notwithstanding any other provision of this Agreement, disclosure of Confidential Information shall not be precluded if such disclosure is in response to a valid order of a court or other governmental body of competent jurisdiction of the United States or any political subdivision thereof or is otherwise required by law or regulation; *provided, however,* that the Receiving Party shall, to the extent practicable, first have given notice to the Disclosing Party and shall have made a reasonable effort to obtain a protective order requiring that the Confidential Information so disclosed be used only for the purposes for which the order was issued or the law or regulation required or to seek other confidential treatment of such information.

5.4 Publication. The parties acknowledge and agree that the SMA research community and medical professionals treating SMA patients will benefit from disclosure of the Data as soon as practicable. Accordingly, should the Foundation wish to publish any Confidential Information contained in a Research Report, it shall provide Company with [**] days' advance notice of such publication (the ***"IP Filing Period"***) to allow Company to file

patent applications covering the Company Technology disclosed in such Research Report; *provided, however,* that at Company's reasonable request, the IP Filing Period shall be extended for an additional [**] days if necessary for the filing of appropriate patent applications covering Company Technology disclosed in or apparent from such Research Report. During the IP Filing Period, the Foundation shall maintain as confidential the Data and the Research Report provided to Foundation by Company. Notwithstanding the foregoing, in no event shall Foundation disclose the structures of any chemical compound being researched or developed by Company in any publication or other public forum without the prior written consent of Company. Except as expressly set forth in this Agreement, the Foundation shall not have the right to use the Data to develop, commercialize, market or sublicense any commercial offering of any product or service based on the Data. The Company shall provide in each [**] Research Report a summary section which is suitable for immediate public disclosure and the Foundation may release copies of such portions of each Research Report and supporting Data to any Third Party investigator who requests such material from the Foundation in writing; *provided, however,* that said Third Party investigator first executes Company's non-disclosure agreement (it being understood that such non-disclosure agreement will not prohibit said Third Party investigator from applying his or her knowledge of the Data to further SMA research and/or to treatment of SMA patients, but will prohibit him or her from transferring such Data except as incidental and necessary to treating SMA patients). The Foundation will treat all other Data in each Research Report as Company Confidential information. To the extent that any journal or other forum in which the Foundation proposes to publish or disseminate the Data requires the authorship or participation of one or more Company employees or contractors who participated in the Research or in the development of a Drug Candidate or Product, Company shall use commercially reasonable efforts to cause such individuals to cooperate with the Foundation in making such publication and, as necessary or appropriate, to be named as authors (or co-authors) of such publication. Any publication or presentation of Data in any Research Report shall acknowledge each party's contribution thereto in accordance with customary scientific practice.

5.5 Publicity; Regulatory Disclosures. It is understood that the parties intend to issue a joint press release announcing the execution of this Agreement, and the parties agree that each party may desire or be required to issue subsequent press releases or make disclosures in regulatory filings relating to this Agreement or activities hereunder. The parties agree to consult with each other reasonably and in good faith with respect to the text and timing of such press releases or other disclosures prior to the issuance thereof, provided that a party may not unreasonably withhold consent to such releases or disclosures, and that either party may issue such press releases or disclosures as it determines, based on advice of counsel, are reasonably necessary to comply with laws or regulations or for appropriate market disclosure. In addition, following the initial joint press release announcing this Agreement, either party shall be free to disclose, without the other party's prior written consent, the existence of this Agreement, the identity of the other party and those terms of the Agreement which have already been publicly disclosed in accordance with this Section 5.5.

6. OWNERSHIP AND USE OF DATA AND INTELLECTUAL PROPERTY.

6.1 Ownership; Reversionary License.

(a) Data. Company shall solely own all data generated as a result of the Research (the ***"Data"***).

(b) Company Technology. Company shall solely own all Company Technology.

(c) Reversionary Licenses to Data and Company Technology. With respect to Research Projects in which [**], in the event that:

(i) Company elects not to continue the Research or subsequent development of at least one Drug Candidate or Product relating to any Research Project in the Field; or

(ii) Company fails to use commercially reasonable efforts to conduct development and commercialization of at least one commercially viable Drug Candidate arising in the Field, and is unable to remedy such failure to comply within [**] days after notice thereof from the Foundation; or

(iii) Company is otherwise in material breach of this Agreement with respect to such Research Project and is unable to remedy such breach within [**] days after notice of such breach from the Foundation;

then, in any such case, Company shall, and it hereby does, grant to the Foundation an exclusive worldwide license, including the right to grant sublicenses, under any Company Technology resulting from such Research Project that relates to a pharmaceutical preparation, composition of matter, method of manufacture and/or method of use of such Drug Candidate Lead Candidates, Drug Candidates and Products in the Field, solely for the purpose of researching, developing, making, having made, using, selling, having sold, offering for sale and importing Drug Targets, Lead Candidates, Drug Candidates and Products in the Field (each such license with respect to a particular Research Project being referred to herein as a ***"Reversionary License"***), and use of such Data by the Foundation or its sublicensee(s) as reasonably necessary or appropriate to exploit such Reversionary License shall not represent a violation of Section 5.1 above; *provided, however,* that in the case of Research Project B such license shall not be granted if (x)

the Company project team, with the concurrence of the Foundation or its advisors, determines that the compounds identified in the conduct of Research Project B are not more active than the [**] in the applicable assay(s), or are more active but [**] for [**], and Company does not pursue development and commercialization of such compounds; or (y) the Foundation chooses not to [**] of Research Project B [**]; and *provided, further,* that the Reversionary License with respect to a particular Research Project shall not become effective (I) if the parties mutually agree, after good faith discussions based on [**] of such Research Project, that such Research Project [**], (II) Company [**] in such Research Project that [**], or (III) Company [**] in such Research Project that [**] but the [**] of the research for such Research Project. If the Reversionary License covers a Product which, as of the date of effectiveness of the Reversionary

License, has [**], and the Reversionary License was granted pursuant to Section 6.1(a)(i), then Foundation [**] a [**] of such Product in the [**] such Product [**] in [**]. In any [**] in the [**], the Reversionary License [**].

(d) Research Tools. The parties acknowledge that the SMA research and clinical communities will benefit from the availability of Research Tools. Company agrees to use commercially reasonable efforts to make Research Tools Controlled by it available to members of the spinal muscular atrophy research and clinical communities (excluding for-profit entities engaged in pharmaceutical research and development) for research or educational purposes on commercially reasonable terms as promptly as practicable following request by the Foundation or such person (it being understood that neither Company nor its corporate partners shall charge reach-through royalties with respect to drugs discovered by such persons using Research Tools, so long as such drugs themselves are not covered by Company Technology); *provided, however,* that Company shall not have any obligation to provide such access before the publication of patent applications containing claims (adequately supported by written description) that cover the relevant Research Tool. Notwithstanding the foregoing, if Company believes in good faith that [**], then Company shall so notify the Foundation in writing, and the parties shall discuss in good faith how to proceed.

6.2 Patent Filings. (a) Company shall file, prosecute and maintain all Patents on the Company Technology at its sole expense. Notwithstanding the foregoing, if Company is obligated to make the Reversionary License to the Foundation as described in Section 6.1(c) above, then the Foundation shall have the right, itself or through its designee, to file, prosecute and maintain Patents licensed under the Reversionary License at its sole expense; *provided, however,* that if [**], and [**], and further provided, that the Company shall have reasonable rights of comment and consultation on all such prosecution and maintenance activities, (b) Each of Company and Foundation shall execute all papers and instruments, and require its employees and contractors to execute all papers and instruments, so as to enable the other party to exercise the rights set forth in Section 6.2(a).

6.3 SMA Research Tools. Foundation shall use commercially reasonable efforts to assist Company in obtaining favorable licensing terms for access to SMA Research Tools necessary or useful for the conduct of the Research.

6.4 No Other License. Other than any license granted pursuant to Section 6.1(c), no license is granted or implied with respect to any Company Technology or Data for any use.

7. TERM; TERMINATION.

7.1 Term. The term of this Agreement shall commence on the Effective Date and shall continue until expiration of the Research Term, unless this Agreement is earlier terminated in accordance with this Article 7.

7.2 Termination for Cause. Each party shall have the right to terminate this Agreement upon 60 days' prior written notice to the other upon the occurrence of any of the following:

(a) Upon or after the bankruptcy, insolvency, dissolution or winding up of the other party (other than a dissolution or winding up for the purpose of reorganization); or

(b) Upon or after the breach of any material provision of this Agreement by the other party if the breaching party has not cured such breach within the 60-day period following written notice of termination by the non-breaching party.

7.3 Termination Upon Principal Scientist's Unavailability. The Foundation may terminate this Agreement upon 30 days' prior written notice to Company in the event the Foundation and Company are unable to agree upon a suitable replacement for the Principal Scientist pursuant to Section 2.3; *provided, however,* that termination in accordance with this Section 7.3 will not trigger the grant of any Reversionary License under Section 6.1. In the event of a termination of this Agreement pursuant to this Section 7.3, and notwithstanding any other provision of this Agreement to the contrary (including but not limited to

Section 7.4), only the provisions of Sections 6.1(a), 6.1(b), 6.2(b), this Section 7.3, the first sentence of Section 6.2(a), and Articles 1, 5, 8, and 9 will survive such termination.

7.4 Consequences of Expiration or Termination. Expiration or termination of this Agreement will not relieve the parties of any obligation accruing prior to such expiration or termination (including, without limitation, any accrued obligation of the Foundation to make payments pursuant to Section(s) 4.1 and/or 4.2). Except as otherwise provided in Section 7.3, and notwithstanding any other provision of this Agreement to the contrary, the provisions of Sections 4.3, 4.4, 4.5, 4.6, 4.7, 7.4, and 7.5, and Articles 1, 5, 6 (to the extent applicable), 8 and 9 will survive expiration or termination of this Agreement.

7.5 Rights in Bankruptcy. All rights and licenses granted under or pursuant to this Agreement by Company are, and will otherwise be deemed to be, for purposes of Section 365(n) of the U.S. Bankruptcy Code, licenses of right to "intellectual property" as defined under Section 101 of the U.S. Bankruptcy Code. The parties agree that the Foundation, to the extent it receives a Reversionary License pursuant to Section 6.1(c), as licensee of such rights under this Agreement, will retain and may fully exercise all of its rights and elections under the U.S. Bankruptcy Code. The parties further agree that, in the event of the commencement of a bankruptcy proceeding-by or against Company under the U.S. Bankruptcy Code, the Foundation will be entitled to a complete duplicate of (or complete access to, as appropriate) any such intellectual property and all embodiments of such intellectual property, and same, if not already in the Foundation's possession, will be promptly delivered to the Foundation (a) upon any such commencement of a bankruptcy proceeding upon its written request therefor, unless Company elects to continue to perform all of its obligations under this Agreement, or (b) if not delivered under clause (a) above, following the rejection of this Agreement by or on behalf of Company upon written request therefor by the Foundation.

8. INDEMNIFICATION.

8.1 Indemnification by Company. Company hereby agrees to save, defend, indemnify and hold harmless the Foundation, its trustees, officers, employees and agents (each, a ***"Foundation Indemnitee"***) from and against any and all losses, damages, liabilities, expenses and costs, including reasonable legal expenses and attorneys' fees ***("Losses"***), to which a Foundation Indemnitee may become subject as a result of any claim, demand, action or other proceeding by any Third Party to the extent such Losses arise directly or indirectly out of (a) the development, manufacture, handling, storage, sale or other disposition of any Drug Candidate or Product by Company, its Affiliate(s) or Licensee(s), or (b) the breach of this Agreement by Company or the gross negligence or willful misconduct of Company, except in each case to the extent such Losses result from (x) the breach of this Agreement by the Foundation or the gross negligence or willful misconduct of any Foundation Indemnitee, or (y) the activities of the Foundation or its agents or employees in connection with any Research Project or related Drug Candidate or Product after the Foundation has received a Reversionary License in connection with such Research Project under Section 6.1(c) ("Reversionary License Activities").

8.2 Conditions to Indemnification. The obligations of Company under Section 8.1 are conditioned upon the Foundation's delivery of written notice to Company of any potential Losses promptly after the Foundation becomes aware of such potential Losses. Company shall have the right to assume the defense of any suit or claim related to the Losses if it has assumed responsibility for the suit or claim in writing. If Company defends the suit or claim, the Foundation may participate in (but not control) the defense thereof at its sole cost and expense.

8.3 Settlements. Neither party may settle a claim or action related to any Losses subject to indemnification under Section 8.1 without the consent of the other party, if such settlement would impose any monetary obligation on the other party or require the other party to submit to an injunction or otherwise limit the other party, its Affiliates, trustees, employees, agents, officers or directors.

8.4 Insurance. During any period when Company, its Affiliate or any Licensee is clinically developing or commercializing any Drug Candidate or Product and for [**] years thereafter, Company, at its own expense, shall maintain clinical trial and/or product liability insurance, as applicable, in an amount consistent with industry standards and only if available on commercially reasonable terms, and shall name the Foundation as an additional insured with respect to such insurance, with respect to losses arising out of or related to the activities contemplated under this Agreement. Company shall provide a certificate of insurance evidencing such coverage to the Foundation upon request.

8.5 Liability of the Foundation. The Foundation assumes any and all risk of personal injury and property damage attributable to the practice by the Foundation, its trustees, officers, employees or agents, or its designee or sublicensee of any license granted by Company to the Foundation hereunder, the breach of this Agreement by the Foundation or any Foundation Indemnitee, or the gross negligence or willful misconduct of any Foundation Indemnitee. Furthermore, the Foundation assumes any and all risk of Losses (as defined above) in connection with any Reversionary License Activities.

9. MISCELLANEOUS.

9.1 Assignment. Except as expressly provided hereunder, neither this Agreement nor any rights or obligations hereunder may be assigned or otherwise transferred by either party without the prior written consent of the other party (which consent shall not be unreasonably withheld). Notwithstanding the foregoing, the Foundation shall have the right to assign or transfer any or all of its rights or obligations under this Agreement to a Third Party that is a non-profit organization upon written notice to Company, provided that the Foundation shall remain liable for any payment obligations accruing hereunder to the extent that such Third Party does not comply with such obligations. Company shall have the right to assign or transfer any or all of its rights or obligations under this Agreement to a Third Party in connection with the transfer or sale of all or substantially all of the portion of Company's business to which this Agreement relates, or in the event of Company's merger or consolidation or change in control or similar transaction or the creation of a special purpose corporation or research and development limited partnership or a joint venture. The rights and obligations of the parties under this Agreement shall be binding upon and inure to the benefit of the successors and permitted assigns of the parties. Any assignment not in accordance with this Agreement shall be void.

9.2 Force Majeure. Neither party shall be held liable or responsible to the other party nor be deemed to have defaulted under or breached this Agreement for failure or delay in fulfilling or performing any term of the Agreement when such failure or delay is caused by or results from causes beyond the reasonable control of the affected party, including, without limitation, fire, floods, earthquakes, natural disasters, embargoes, war, acts of war (whether war be declared or not), insurrections, riots, civil commotions, strikes, lockouts or other labor disturbances, acts of God or acts, omissions or delays in acting by any governmental authority or the other party.

9.3 Governing Law. This Agreement shall be governed by, and construed and enforced in accordance with, the laws of the State of New York, without regard to its choice of law provisions; provided, however, that with respect to intellectual property filings, such filings will be governed by the federal laws of the United States, or, if outside the United States, by the applicable intellectual property laws of the relevant jurisdiction(s).

9.4 Waiver. Except as specifically provided for herein, the waiver from time to time by either party of any right or failure to exercise any remedy shall not operate or be construed as a continuing waiver of the same right or remedy or of any other of such party's rights or remedies provided under this Agreement.

9.5 Severability. In case any provision of this Agreement shall be invalid, illegal or unenforceable, the validity, legality and enforceability of the remaining provisions shall not in any way be affected or impaired thereby.

9.6 Independent Contractors. It is expressly agreed that Company and the Foundation shall be independent contractors and that the relationship between the two parties shall not constitute a partnership, joint venture or agency of any kind. Neither party shall have the authority to make any statements, representations or commitments of any kind, or to take any

action, which shall be binding on the other party, without the prior written consent of the other party.

9.7 Notices. All notices and other communications provided for hereunder shall be in writing and shall be mailed by first-class, registered or certified mail, postage paid, or delivered personally, by overnight delivery service or by facsimile, with confirmation of receipt, addressed as follows:

If to the Foundation: Spinal Muscular Atrophy Foundation
1776 Broadway, 22nd Floor
New York, NY 10019
Fax: (212) 247-3079
Attention: Ms. Cynthia Joyce, Executive Director

With a copy to: Cooley Godward LLP
4401 Eastgate Mall
San Diego, CA 92121
Fax: (858) 550-6420
Attention: Jane K. Adams, Esq.

If to Company: PTC Therapeutics, Inc.
100 Corporate Court
South Plainfield, NJ 07080-2449
Fax: 908-222-7231

Attention: Mark Boulding, Senior Vice President and
General Counsel

With an email copy to: legal@ptcbio.com

Either party may by like notice specify or change an address to which notices and communications shall thereafter be sent. Notices sent by facsimile shall be effective upon confirmation of receipt, notices sent by mail or overnight delivery service shall be effective upon receipt, and notices given personally shall be effective when delivered.

9.8 Entire Agreement; Amendment. This Agreement (including the Exhibits hereto, as such Exhibits may be amended from time to time by mutual written agreement of the parties) sets forth all of the agreements and understandings between the parties hereto with respect to the subject matter hereof, and supersedes and terminates all prior agreements and understandings between the parties with respect to the subject matter hereof. There are no other agreements or understandings with respect to the subject matter hereof, either oral or written, between the parties. Except as expressly set forth in this Agreement, no subsequent amendment, modification or addition to this Agreement shall be binding upon the parties hereto unless reduced to writing and signed by the respective authorized officers of the parties.

9.9 Headings; Section References. The captions contained in this Agreement are not a part of this Agreement, but are merely guides or labels to assist in locating and reading the

several Articles and Sections hereof. Section references herein are to the corresponding Sections of this Agreement unless otherwise indicated.

9.10 Counterparts. This Agreement may be executed in counterparts, each of which shall be deemed an original, but all of which together shall constitute one and the same instrument.

[REMAINDER OF THIS PAGE INTENTIONALLY LEFT BLANK]

IN WITNESS WHEREOF, the parties hereto have executed this Agreement as of the Effective Date.

SPINAL MUSCULAR ATROPHY FOUNDATION	**PTC THERAPEUTICS INC.**
By: /s/ Loren A. Eng	By: /s/ Stuart Peltz
Printed Name: Loren A. Eng	Printed Name: Stuart Peltz
Title: President	Title: President and CEO

[SIGNATURE PAGE TO SPONSORED RESEARCH AGREEMENT]

EXHIBIT A

RESEARCH PLAN

PTC proposes to collaborate with the SMA foundation in order to identify new therapeutics for the treatment of spinal muscular atrophy (SMA). PTC will utilize its expertise, its platform technologies, and compounds to identify new drugs to treat SMA. Three programs for the discovery and development of drugs to treat SMA are contemplated:

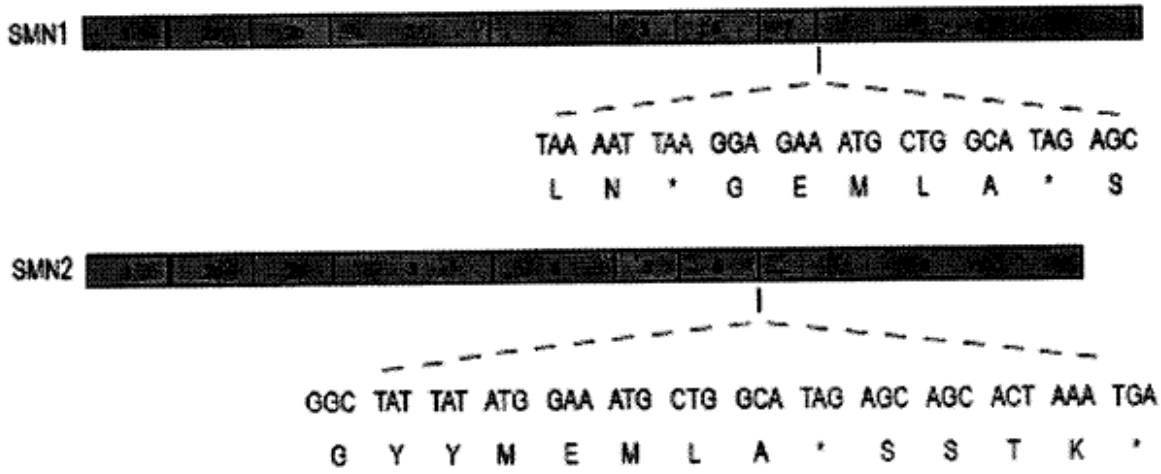
[**].

Overview of SMA

Spinal muscular atrophy (SMA) is a common autosomal recessive neurodegenerative disease characterized by degeneration of motor neurons of the anterior horn of the spinal cord resulting in muscle weakness and atrophy. SMA can be

subdivided into three clinical groups on the basis of age of onset and severity of the symptoms. The acute type I form is characterized by severe, generalized muscle weakness and hypotonia that is seen either at birth or within the first 3 months of life. Death from respiratory failure usually occurs within the first 2 years. Type II children are able to sit, although they cannot stand or walk unaided. They suffer significant respiratory morbidity and earlier mortality. Type III patients have proximal muscle weakness, starting after age of 2. They generally have a milder course, with the potential for normal life expectancy.

SMA results from reduced expression of survival motor neuron protein (SMN). The SMN gene is duplicated as an inverted repeat on human chromosome 5. The telomeric copy of SMN (SMN1) is deleted or mutated in over 98% of SMA patients. These patients retain at least one copy of the centromeric SMN gene called SMN2. The SMN2 gene has a mutation such that SMN2-derived transcripts are alternatively spliced and encode a truncated protein lacking exon 7 (SMNDExon7). The number of SMN2 copies strongly correlates with severity of SMA.



The quantity of a particular protein synthesized in a given time depends on both the cellular concentration of its mRNA and how efficiently the mRNA is used by cellular

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translational apparatus. Multiple cellular mechanisms that affect mRNA availability or utilization are major regulators of protein production and are known as post-transcriptional control processes. PTC targets these processes in its drug discovery efforts. The mRNA sequences that are often found the non-coding regions, known as “untranslated regions” or UTRs. The region before the protein coding region is known as the 5’-UTR, while the region following the protein coding region is known as the 3’-UTR. The largest number of known post-transcriptional control determinants map to the 5’- and 3’-untranslated regions of an mRNA. Post-transcriptional regulation occurs through interaction of cellular factors with sequence elements including secondary structures, protein-binding sites, upstream open reading frames, internal ribosome entry sites, and poly(A) tail.

Examination of the 5’ and 3’ untranslated regions of SMN2 mRNA reveals a number of sequence elements that strongly suggest that the SMN2 mRNA is post-transcriptionally regulated. The 5’ UTR of SMN2 contains a regulatory element known as an upstream open reading frame (uORF). Several lines of evidence strongly suggest that uORFs in the 5’ UTRs of mRNAs regulate gene expression by modulating efficiency of translation and mRNA stability. The SMN2 5’ UTR also contains 63% of C and G nucleotides suggesting a high degree of secondary structure, which can be an excellent platform for binding of proteins that regulate ribosome scanning and, therefore, translation efficiency. The 3’ UTR of SMN2 is 559 nucleotides in length and contains several conserved elements that serve as protein binding sites that are involved in regulating translation efficiency and mRNA stability.

The GEMS technology platform

The GEMS technology platform (Gene Expression Modulation by Small-molecules) targets the post-transcriptional control mechanisms in order to identify small molecules that alter gene expression to increase or decrease protein levels. Compounds identified by the GEMS technology modulate the post-transcriptional control mechanisms of gene expression that act through the 5’- 3’-untranslated regions of mRNA. The GEMS technology can be applied to the SMN2 gene to identify small molecules that increase the expression of the SMN2 gene.

Overview of program [**]

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of four pages were omitted. [**]

Exhibit B

RESEARCH MILESTONES

Milestone 1: [**]

Milestone 2: [**].

Milestone 3: [**].

Goal: A set of compounds characterized from all three Research Projects.

Final

AMENDMENT No. 1 TO SPONSORED RESEARCH AGREEMENT

This first amendment ("First Amendment") to the Sponsored Research Agreement is entered into as of the 12th day of October, 2007 (the "Amendment Effective Date"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. ("PTC"), with reference to the following facts and circumstances.

WHEREAS Foundation and PTC are parties to that certain Sponsored Research Agreement dated as of June 1, 2006 (the "Agreement");

WHEREAS PTC has achieved all the initial milestones set forth in Exhibit B to the Agreement, and Foundation has made the payments associated with such milestones under the Agreement;

WHEREAS, the Parties desire to extend the Agreement to allow additional funding by Foundation in connection with continued research focused on small molecule therapeutics for SMA;

NOW THEREFORE, in consideration of the premises and mutual covenants contained in this First Amendment, the Parties agree as follows:

1. Definitions. Except as expressly set forth herein, all capitalized terms used herein and not otherwise defined shall be as defined in the Agreement

2. Additional Research. The Parties agree to the modification of the Research Plan attached as Exhibit A-1 to allow for PTC to perform early structure-activity relationship work on Hits identified under each of the three Research Projects (the "Additional Research"). The goal of such research will be the presentation by PTC of a list characterized Lead Candidates for further discussions with Foundation with respect to prioritization and potential funding of Lead Optimization by Foundation. The expected duration of such research is [**] months. The Foundation shall have the exclusive option (the "Option") to continue funding development of all Lead Candidates presented by the company at the end of the Amendment Term (as defined below) through the identification of a Drug Candidate suitable for an IND filing, subject to the following terms and conditions: (a) the Foundation may exercise the Option by providing written notice to PTC of its intent to so fund development within [**]days following the date on which the Final Report (as defined below) is transmitted to Foundation; (b) following such written notice, the Parties shall negotiate in good faith for a period of no longer than [**] days the budget and terms and conditions of such proposed funding, and (c) if the Parties are unable to reach agreement within such [**] day period, then Foundation's rights under the Option shall expire. Notwithstanding the foregoing, in no case shall the Option preclude PTC from entering into partnering arrangements or other agreements with commercial partners with respect to the Research Projects, so long as PTC is in compliance with the other terms of the Agreement and this Amendment.

PTC shall conduct such Additional Research in accordance with the terms of the Agreement as amended herein, including but not limited to PTC's obligations under Section 2.5 of the Agreement (captioned "Performance Standards"). In connection with such Additional Research,

the Research Term shall be extended, without interruption, until the date which is eight (8) months following the Amendment Effective Date (the "Amendment Term").

3. Research Reports. In lieu of the Research Reports that would otherwise be due from PTC under Section 2.7 of the Agreement during the Research Term, PTC shall make the following reports: (a) [**] months following the Amendment Effective Date, a summary report showing progress with respect to the Additional Research and identifying any limiting factors or other considerations that may affect completion of the Additional Research (the "Mid-Stage Report"), and (b) within [**] days of completion of the Additional Research, a final report containing the recommendations by PTC for selection of compounds for further research and potential Lead Optimization (the "Final Report"). In addition, PTC will make itself available for Research Team conference calls following its internal research update meetings, which are expected to occur every [**] weeks, for informal discussion of the program.

4. Additional Payments by Foundation. Foundation shall pay PTC a total of [**] US dollars ($[**]) in partial support for the Additional Research as follows: (a) [**] US dollars ($[**]) within [**] days of the Amendment Effective Date; (b) [**] US dollars ($[**]) within [**] days of the receiving the Mid-Stage Report; and (c) [**] US dollars ($[**]) within [**] days of the receiving the Final Report.

5. Foundation Negotiation Rights. During the Research Term and any subsequent extension of the collaboration and for the [**] month period thereafter, before entering into any written agreement with any third party under which PTC is obligated to conduct screening of its library for small molecules that modulate the expression of Drug Targets in exchange for funding, PTC shall first conduct good faith negotiations with Foundation with respect to provision of such funding by Foundation. Notwithstanding the foregoing, PTC's obligations under this First Amendment Section 5 shall neither prohibit nor in any way limit (a) PTC's ability to fulfill contractual commitments to third parties in effect as of the Amendment Effective Date, (b) PTC's ability to enter into license agreements or otherwise collaborate with third parties with respect to compounds or programs directed against SMA developed by such third parties; nor (c) PTC's ability to enter into any agreements or arrangements with respect to modulation of genes relevant to SMA via nonsense suppression.

6. Coordination of Funding. During the Research Term and any subsequent extension of the collaboration and for the [**] month period thereafter, should PTC require additional funds for the conduct of any Research Project, the Foundation will be consulted prior to any fundraising efforts for such Research Project. Should PTC identify an opportunity for agreement with any third party or parties with respect to additional or continued funding specifically directed to Research Projects, it will provide reasonable advance notice to Foundation, and the parties will negotiate in good faith (involving such third party or parties as appropriate) to develop a structure that supports such additional funding, based on the following principles: (a) entities co-funding a Research Project should share information on the Research with each other, subject to appropriate confidentiality provisions, (b) governance with respect to co-funded Research Projects should be via a joint steering committee including representatives of Foundation, PTC, and any third parties, (c) within the steering committee for a particular co-funded Research Project, role in decision-making with respect to matters within the sole purview of funding

entities (including but not limited to strategic discussions as outlined in section 2.4 of the Agreement) should be [**], and (d) entities that have provided funding to a co-funded Research Project should have an opportunity (subject to compliance with the terms of their respective funding agreements) to continue their support of such Research Project. For clarity, PTC's obligations under this First Amendment Section 6 shall in no way limit PTC's ability to engage in general fund-raising activities and to enter into agreements relating thereto.

7. No Conflicts. Each Party represents and covenants that (a) it has the authority and right, to enter into this First Amendment and to perform its obligations with respect to the Additional Research, and (b) during the Research Term and any subsequent extension of the collaboration and for the [**] month period thereafter, it will not enter into any agreement with any third party that would conflict with the performance of its obligations hereunder, or with Foundation's potential funding of Lead Optimization on terms mutually acceptable to the parties.

8. Notices. The address of the Foundation for the purposes of section 9.7 of the Agreement shall be as follows:

Spinal Muscular Atrophy Foundation
888 Seventh Avenue
Suite 400
New York, NY 10019
Fax: 212-347-2079
Attention: Ms. Cynthia Joyce, Executive Director

With a copy to Cooley Godward LLP as currently provided in the Agreement.

9. No Other Modifications. In all other respects, the terms and conditions of the Agreement shall remain unchanged and in full force and effect. In the event of any conflict between the terms of this First Amendment and the terms of the Agreement, the terms of this First Amendment shall govern.

IN WITNESS WHEREOF, the Parties have executed this First Amendment by their duly authorized officers as of the date set forth above.

PTC THERAPEUTICS, INC.	**SPINAL MUSCULAR ATROPHY FOUNDATION**
/s/ Mark E. Boulding	/s/ Loren Eng
By: Mark E. Boulding	By: Loren Eng
Title: Senior Vice President and General Counsel	Title: SMA Foundation, President

EXHIBIT A-1

RESEARCH PLAN FOR ADDITIONAL RESEARCH

The research goal of this modification to the Research Plan is to complete hit characterization of the active compounds from Project A, Project B, and Project C (the "Projects"), to perform in vitro pharmaceutical profiling of leads from these Projects, to delineate clearly a screening tier to meet the development candidate goal for these Projects, and to identify directions for the full optimization process.

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of one page was omitted. [**]

Execution Version

AMENDMENT No. 2 TO SPONSORED RESEARCH AGREEMENT

This second amendment ("Second Amendment") to the Sponsored Research Agreement is entered into as of the 1st day of May, 2009 (the "Second Amendment Effective Date"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. (the "Company"), with reference to the following facts and circumstances.

WHEREAS Foundation and Company are parties to that certain Sponsored Research Agreement dated as of June 1st, 2006, as amended by the First Amendment on October 12th, 2007 (the "Agreement");

WHEREAS, the parties desire to further amend the Agreement to allow additional funding by Foundation in connection with continued research focused on small molecule therapeutics for SMA;

NOW THEREFORE, in consideration of the premises and mutual covenants contained in this Second Amendment, the parties agree as follows:

1. Definitions.

(a) Section 1.5 of the Agreement ("Company Clinical Trial") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Company Clinical Trial' means any human clinical trial of a Development Candidate or Product conducted by or on behalf of Company or its Affiliates or Licensees pursuant to an effective IND submitted by or on behalf of Company or its Affiliates or Licensees."

(b) Section 1.11 of the Agreement ("Data") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Data' means all data generated as a result of the Research or as a result of Company's or its Affiliate's or Licensee's research, Development, or commercialization of Drug Candidates or Products."

(c) Section 1.15 of the Agreement ("Field") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Field' means the treatment, mitigation or prevention of [**]."

(d) Section 1.16 of the Agreement ("First Commercial Sale") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'First Commercial Sale' means the date of the first commercial sale in a country or region by or on behalf of Company or its Affiliate or Licensee of a Product to a Third Party end user in an arm's-length transaction after an NDA has been approved for such Product in such country or region."

(e) Section 1.18 of the Agreement ("IND") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'IND' means an investigational new drug application submitted for action to the FDA or any other similar application submitted for action to an appropriate Regulatory Agency in a country or group of countries other than the United States."

(f) Section 1.23 of the Agreement ("Licensee") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Licensee' means any Third Party to which Company grants rights with respect to any Lead Candidate, Reversion Candidate, Development Candidate or Product in accordance with Second Amendment Section 10."

(g) Section 1.24 of the Agreement ("Net Sales") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Net Sales' means gross amounts received by Company and its Affiliates from Third Parties other than Affiliates or Licensees for sales of a Product to Third Parties other than Affiliates or Licensees (unless such Affiliate or Licensee is the end user of such Product, in which case the amount billed therefor shall be deemed to be the amount that would be billed to a Third Party end user in an arm's-length transaction), less the following deductions, without duplication: (a) actual bad debts actually written off which are attributable to sales of such Product; (b) any rebates, quantity, trade and cash discounts, and other usual and customary discounts to customers granted and taken in the ordinary course of business; (c) retroactive price reductions, allowances, chargebacks, rebates, adjustments and amounts repaid or credited by reason of rejections or returns of such Product (including returns of such Product by reason of a product recall or damaged or defective goods); (d) freight, shipping and insurance charges; (e) distribution, packing, handling and transportation charges for Products to the extent that they are included in the price or otherwise paid by the customer; (f) compulsory payments and rebates, actually paid or deducted; (g) customs duties and other governmental charges, as well as sales, use, excise, inventory, value added, and other taxes (except income taxes), related to the sale of such Product; (h) payments, discounts, rebates, fees, reimbursements or similar payments granted to managed health care organizations or federal, state or local governments, their agencies, purchasers or reimbursers or any government subsidized programs, wholesalers or other distributors, buying groups, health insurance carriers, other institutions, or discount programs; and (i) any write-offs from quantities of such Product donated by Company to Third Parties for charitable or humanitarian purposes, to the extent included in gross sales. The foregoing adjustments shall be consistent with customary accounting practices within Company (or its respective Affiliates) and in accordance with U.S. Generally Accepted Accounting Principles or with a similar internationally-accepted accounting standard, consistently applied."

(h) Section 1.38 of the Agreement ("Reversionary License") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Reversionary License' shall have the meaning set forth in Section 6.1(c)(2)(i) of the Agreement."

(i) Section 1.42 of the Agreement ("Third Party Patent License") shall, as of the Second Amendment Effective Date, be amended and restated as follows: "'Third Party Patent License' shall have the meaning provided in Section 3.4(c)."

(j) The following defined terms shall apply as of the Second Amendment Effective Date:

"AAA" shall have the meaning set forth in Second Amendment Section 17(b).

"Appointing Party" shall have the meaning set forth in Second Amendment Section 5(h).

"Baseball Arbitration" shall have the meaning set forth in Second Amendment Section 17(a).

"Benchmark Trigger" shall have the meaning set forth in Section 3.3(b) of the Agreement.

"Buy-Out Notice" shall have the meaning set forth in Section 3.3(b)(v) of the Agreement.

"Buy-Out Right" shall have the meaning set forth in Section 3.3(b)(iv) of the Agreement.

"Chief Executive Officer" means (a) the person holding the title of Chief Executive Officer of a party at the time in question or (b) if there is no person holding the title of Chief Executive Officer of a party at the time in question, then the person holding the title of Chairman of the Board of Directors of such party at such time.

"Collaboration Activities" means direct efforts by Company or its agents to pursue any proposal related to a license, option, joint venture, collaboration, sale or other strategic transaction (other than a PTC Corporate Change) involving the DC Research or any Lead Candidate, Reversion Candidate, Development Candidate or Product, but excluding [**] entered into with a Third Party under which Company remains primarily responsible for Development and commercialization of Lead Candidates, Reversion Candidates, Development Candidates and Products. For clarity, activities routinely performed by Company's business development team to promote Company's general drug discovery and development capabilities (including discovery research in the Field) shall not constitute Collaboration Activities.

"Commercially Reasonable Efforts" means:

(a) with respect to the efforts to be expended by a party with respect to any objective, except as otherwise provided in clause (b) below, such reasonable, diligent and good faith efforts as such party [**]; and

(b) [**].

"Company Indemnitee" shall have the meaning set forth in Second Amendment Section 13(d).

"Company Losses" shall have the meaning set forth in Second Amendment Section 13(d).

"Corrective Plan" shall have the meaning set forth in Second Amendment Section 2(g)(1).

"Cost/Timeline Issue" shall have the meaning set forth in Second Amendment Section 2(g).

"DC Research" shall have the meaning set forth in Second Amendment Section 2(a).

"DC Timeline Goal" shall have the meaning set forth in Second Amendment Section 2(a).

"Development" means, with respect to a Drug Candidate, Development Candidate, or Product, all non-clinical (including preclinical) research/development, clinical research/development, and related activities directed to obtaining Regulatory Approval of such Drug Candidate, Development Candidate, or Product, including but not limited to clinical trials, toxicology studies, drug metabolism and pharmacokinetics (DMPK) studies, statistical analysis and report writing, clinical trial design and operations, preparing and submitting INDs and applications for Regulatory Approval, activities related to development and optimization of a commercial-grade manufacturing process and formulation for such Drug Candidate, Development Candidate, or Product, safety reporting, data management and all regulatory affairs and project management related to the foregoing. When used as a verb, "Develop" means to engage in Development.

"Development Candidate" or "DC" means, on a Research Project-specific basis, a Drug Candidate that the JSC formally declares meets criteria established by the JSC indicating such Drug Candidate is suitable for progression to IND-enabling pre-clinical studies in support of future human clinical trials.

"Development Deadline Document" shall have the meaning set forth in Section 3.1 of the Agreement.

"Development Election Notice" shall have the meaning set forth in Second Amendment Section 3(d).

"Development Plan" shall have the meaning set forth in Section 3.1 of the Agreement.

"Enrollees" shall have the meaning set forth in Second Amendment Section 13(b)(2).

"JSC" shall have the meaning set forth in Second Amendment Section 5(a).

"GLP Research" shall have the meaning set forth in Second Amendment Section 3(a).

"GLP Toxicology Studies" shall have the meaning set forth in Section 3.2 of the Agreement.

"Licensee Data" shall have the meaning set forth in Second Amendment Section 10(d)(ii).

"Licensee Technology" shall have the meaning set forth in Second Amendment Section 10(d)(ii).

"M&A Approval Request" shall have the meaning set forth in Second Amendment Section 9(a).

"M&A Certification" shall have the meaning set forth in Second Amendment Section 9(b)(4).

"M&A Notice" shall have the meaning set forth in Second Amendment Section 9(b).

"NDA" means a new drug application approved by the FDA or any other similar application approved by the appropriate Regulatory Agency in a country or group of countries other than the United States."

"Non-DC Research" shall have the meaning set forth in Second Amendment Section 18(a).

"Option Period" means the period commencing upon the end of the [**] day period set forth in Second Amendment Section 3(d) and ending [**] years later; provided, however that such period shall be extended for [**] if Foundation pays Company [**] US dollars ($[**]) and for a [**] if Foundation makes a [**] US dollar ($[**]) payment to Company.

"Partnering Notice" shall have the meaning set forth in Second Amendment Section 10(d).

"Patients" shall have the meaning set forth in Second Amendment Section 13(c)(1).

"[**]" shall have the meaning set forth in Section 4.3(a) of the Agreement.

"Phase 1 Clinical Trial" means any human clinical study of a Product that is intended as initial clinical safety testing in healthy volunteers or a limited patient population, or studies directed toward understanding the mechanisms or metabolism of the Product.

"Phase 2 Clinical Trial" means any human clinical study of a Product subsequent to a Phase 1 Clinical Trial and prior to a Pivotal Clinical Trial that is intended to study the safety, dosage and initial efficacy in a limited patient population, and is prospectively designed to support the continued testing of the Product in one or more further Phase 2 Clinical Trials or in a Pivotal Clinical Trial.

"Pivotal Clinical Trial" means a pivotal human clinical study of a Product that is prospectively designed to confirm with statistical significance in an expanded patient population the efficacy and safety of a drug in a given patient population, and the results of which are intended to form the basis for Regulatory Approval. For the avoidance of doubt, a clinical trial that meets the foregoing criteria shall be deemed a Pivotal Clinical Trial regardless of whether it is characterized as a "Phase 2b," or "Phase 2b/3," or "Phase 3" clinical trial.

"Proof-of-Concept" means, with respect to a particular Development Candidate, (a) the initiation of a Pivotal Clinical Trial for the treatment, mitigation or prevention of SMA or, with the written consent of the Foundation, any other disease, indication or medical condition or (b) if sooner, the submission of an application for Regulatory Approval for the use of such Development Candidate to treat, mitigate or prevent SMA or, with the written consent of the Foundation, any other disease, indication or medical condition.

"Proposals" shall have the meaning set forth in Second Amendment Section 17(d).

"[**]" shall have the meaning set forth in Section 3.4(a) of the Agreement.

"PTC Corporate Change" means (a) a merger, consolidation, amalgamation, share exchange, business combination, issuance of securities (other than Company's initial public offering registered on Form S-1 (or any successor form) under the Securities Act of 1933, as amended, and the rules promulgated thereunder), acquisition of securities, reorganization, recapitalization, tender offer, exchange offer or other similar transaction as a result of which either (i) Company's stockholders immediately prior to such transaction in the aggregate cease to own at least 50% of the voting shares of the entity surviving or resulting from such transaction (or the ultimate parent entity thereof) (where voting refers to being entitled to vote for the election of directors or similar management body of the applicable entity) or (ii) in which a Third Party or "group" (as defined in the Securities Exchange Act of 1934, as amended, and the rules promulgated thereunder) (excluding a "group" consisting of existing stockholders of Company as of the date of this Agreement) directly or indirectly acquires beneficial or record ownership of securities representing 50% or more of Company's voting shares or (b) a sale, lease, exchange, transfer, license, acquisition or

disposition of at least 50% of the assets of Company and its subsidiaries, taken as a whole, in a single transaction or a series of related transactions. For purposes of clarity, a "reverse merger," in which in a transaction or series of related transactions, Company consolidates or merges with another entity and the holders of the outstanding voting shares of Company immediately preceding such consolidation or merger hold more than fifty percent (50%) of the voting shares of the resulting entity, shall not be considered to be a PTC Corporate Change.

"PTC License Income" means all royalties, license fees, milestone payments, annual maintenance fees or similar payments or consideration paid by a Licensee to Company or its Affiliates in consideration for the grant by Company or its Affiliate of a license to develop, make, have made, use, distribute for sale, promote, market, offer for sale, sell, have sold, import or export Drug Candidates or Products or for the practice of such license (with any of the foregoing consideration received by Company other than in the form of cash to be valued at its fair market value as of the date of receipt), provided that PTC License Income shall exclude the proceeds of any debt or equity issuance (except to the extent such payments exceed the fair market value of such securities upon date of receipt, in which event such excess over fair market value shall be included in the calculation of PTC License Income), research and development funding (except to the extent such funding is not reimbursement for the Company's commercially reasonable out-of-pocket, personnel and indirect expenses incurred after the grant of such license to such Licensee and pursuant to a research or development plan approved by such Licensee, in which event such excess shall be included in the calculation of PTC License Income), and any merger or acquisition consideration.

"Publishing Party" shall have the meaning set forth in Section 5.4(a) of the Agreement.

"Regulatory Agency" means, with respect to the United States, the FDA, and, in the case of a country other than the United States, such other appropriate regulatory agency or authority with similar responsibilities.

"Repayment Amount" shall have the meaning set forth in Section 4.3(a) of the Agreement.

"Research Cap" shall have the meaning set forth in Second Amendment Section 2(d).

"Research Compound" shall have the meaning set forth in Second Amendment Section 18(c).

"Research Report" means (a) with respect to any report made prior to the Second Amendment Effective Date, a report defined as such in Section 2.7 of the Agreement or a report made pursuant to First Amendment Section 3, or (b) with respect to any report made on or after the Second Amendment Effective Date, a report described in Second Amendment Section 4(b).

"Reversion Candidate" means (a) each Development Candidate and (b) each Lead Candidate designated as such pursuant to Second Amendment Section 5(b)(vi) or 5(c)(i).

"Reversion Notice" means a notice identified as such in Section 3.2, 3.4(a)(i), 3.4(b), 3.4(c), 3.4(d), or 6.1(c)(1) of the Agreement or Second Amendment Section 3(d).

"Reversion Products" shall have the meaning set forth in Section 6.1(c)(2)(i) of the Agreement.

"Reviewing Party" shall have the meaning set forth in Section 5.4(a) of the Agreement.

"Sales Threshold" shall have the meaning set forth in Section 4.3(b) of the Agreement.

"Second Amendment Term" means the period from the Second Amendment Effective Date until the end of the Research Term.

"Secondary Research Project" shall have the meaning set forth in Second Amendment Section 2(a).

"SMAF Clinical Trials Advisory Committee" shall have the meaning set forth in Second Amendment Section 13(a).

"SMAF Funding Amount" shall have the meaning set forth in Section 4.3(a) of the Agreement.

"Special Termination" shall have the meaning set forth in Second Amendment Section 3.

"Term" shall have the meaning set forth in Section 7.1 of the Agreement.

"Worldwide Net Sales" means the sum of (i) Net Sales and (ii) net sales by Licensees, with such net sales being calculated according to the definition of "Net Sales," but substituting "Licensee" for "Company" as the context requires; provided, however, that if pursuant to a written license agreement with Licensee, Company has agreed to a commercially reasonable definition of net

sales by such Licensee that is reported to Company by Licensee on a quarterly basis, such reported net sales may be used in the calculation of "Worldwide Net Sales."

(k) Except as expressly set forth herein, all capitalized terms used herein and not otherwise defined shall be as defined in the Agreement. For clarity, all definitions of terms that

reference Agreement Sections refer to those Agreement Sections as amended by this Second Amendment.

2. Continuing Research.

(a) The parties agree to the modification of the Research Plan and related budget attached as Exhibit SA-1 to allow for Company to perform continued research activities (the "DC Research") with respect to [**] Research Projects previously funded under the Agreement and the First Amendment. The goal of such research will be the presentation by Company of one (1) Development Candidate from one (1) Research Project for further discussion with Foundation with respect to potential funding of development of such Development Candidate by Foundation, and continuation of one (1) backup program with respect to the other Research Project (the "Secondary Research Project"). The expected duration of the DC Research is [**] months from the Second Amendment Effective Date (the "DC Timeline Goal").

(b) Company shall conduct such DC Research (i) in accordance with Exhibit SA-1, subject to amendment by the JSC as provided in this Second Amendment, and (ii) in accordance with the terms of the Agreement as amended herein, including but not limited to Company's obligations under Section 2.5 of the Agreement (captioned "Performance Standards").

(c) In connection with such DC Research, the Research Term shall be extended, without interruption, until the earliest of (i) the date upon which the JSC first designates a Development Candidate, (ii) the date which is [**] years following the Second Amendment Effective Date or (iii) the effective date of any termination of the Research Term pursuant to Second Amendment Section 3.

(d) The parties will fund the overall total cost of the DC Research based on the Research Plan and related budget attached as Exhibit SA-1, with Foundation contributing approximately [**]% and Company contributing approximately [**]% of such overall total cost of the DC Research as more explicitly specified in such budget, such overall total cost not to exceed $[**] (the "Research Cap") and the Foundation's share of such total cost not to exceed $[**]. During the Research Term, Company will invoice Foundation on a quarterly basis for Foundation's share of the costs incurred in connection with the Research Plan for the preceding calendar quarter, payable within [**] days of receipt by Foundation, subject to Second Amendment Sections 2(d)(i) and 2(d)(ii). Such invoices shall include: (A) an accounting, in reasonable detail sufficient to evaluate performance of the Research Plan by Company, of Company's activities over the applicable period, (B) a breakout of FTEs and other resources allocated to each Research Project and (C) an itemization in reasonable detail of the categories of out-of-pocket costs incurred by Company that are included in such invoice. When invoicing Foundation or developing or presenting any budget related to the Research Plan, Company will in all cases apply the FTE rates specified in Exhibit SA-1 to the applicable category of FTE, and no additions or changes to the FTE categories or rates specified in Exhibit SA-1 shall be made by Company absent prior written consent of Foundation. Company will promptly respond to all requests by Foundation for additional information regarding such out-of-pocket costs. Company's commitment, between [**] and [**], of [**] dollars ($[**]) in funding towards the DC Research shall be available to Company in the form of an invoice credit against Company's share of the cost of the DC Research until expended and shall count towards the Research Cap.

Promptly after the Second Amendment Effective Date, Company will provide Foundation with an invoice for [**] percent ([**]%) of the amount that Company spent between [**] and the Second Amendment Effective Date to perform the DC Research. Such invoice shall include the information specified in (A), (B) and (C) within this Second Amendment Section 2(d) and shall be payable within [**] days of receipt by Foundation. The entire amount paid by Foundation pursuant to such invoice shall count towards the Research Cap and towards Foundation's share of the Research Cap.

(i) Subject to Second Amendment Section 2(d)(ii), Foundation shall not be responsible for its share of any DC Research costs that exceed the budget for any calendar quarter unless:

(1) such costs exceed the budget for such calendar quarter by less than [**] dollars ($[**]) or [**] percent ([**]%) (whichever is less);

(2) such costs exceed the budget for such calendar quarter by more than [**] dollars ($[**]) or [**] percent ([**]%) (whichever is less) but less than [**] percent ([**]%) and Company provided written notice to Foundation prior to incurring such budget overrun; or

(3) such costs exceed the budget for such calendar quarter by more than [**] percent ([**]%) and Foundation approved such budget overrun in writing before it was incurred.

(ii) If at any time during the Second Amendment Term, the total cost incurred in the performance of the DC Research during the period from the Second Amendment Effective Date until the end of the most recent calendar quarter exceeds the cumulative budget for such period by [**] dollars ($[**]) or more, then Second Amendment Section 2(d)(i) shall not apply to any subsequent cost overruns and Foundation shall not be responsible for its share of any additional costs that exceed the applicable budget for any subsequent quarter unless Foundation approved such budget overrun in writing before it was incurred.

(e) Foundation may provide its share of the budget under the Research Plan via other sources of funding, subject to prior agreement of the parties and the existing terms of the Agreement. One hundred percent (100%) of all funds, if any, received by Company during the Second Amendment Term from the Department of Defense directed to the DC Research or the Development of a Development Candidate as a result of the advocacy of the Foundation will count toward the Foundation's share of the costs incurred in connection with the Research Plan; provided, however, that [**] in the [**] pursuant to [**] of the Agreement. At Foundation's request, Company shall promptly complete all paperwork required or reasonably useful to secure receipt by Company of such funds from the Department of Defense.

(f) Company may provide its share of the budget via government grants or grants from nonprofit organizations; provided however, that, except for mandatory licenses and similar or related rights granted to government entities, Company's acceptance of such grants shall not have any effect on Foundation's rights pursuant to this Agreement; further provided, however, that with respect to any nonprofit organizations that have as a specific aspect of their general

mission the funding of research on SMA, Company shall first seek Foundation's written consent and the parties shall negotiate in good faith any required amendments to this Agreement or separate agreements to accommodate grants from such organizations, with the guiding principle that this Agreement remain the primary document governing the conduct of the DC Research by the parties. Company shall use Commercially Reasonable Efforts to obtain additional funding for the Secondary Research Project from government grants or grants from nonprofit organizations (subject to the provisions set forth in the preceding sentence with respect to funding from any nonprofit organizations that have as a specific aspect of their general mission the funding of research on SMA); provided however, that, except for mandatory licenses and similar or related rights granted to government entities, Company's acceptance of such grants shall not have any effect on Foundation's rights pursuant to this Agreement. If Company obtains such funding in an amount that exceeds Company's share of the budget for the Secondary Research Project, Company shall notify Foundation in writing and the JSC shall revise the Research Plan and related budget to reflect the additional work to be performed on the Secondary Research Project with such funds, (i) which additional work shall be under the purview of the JSC and the compounds resulting from such work shall remain Drug Candidates, Reversion Candidates, or Development Candidates, as the case may be, and (ii) which revised budget shall not require the Foundation to contribute any money to pay for or reimburse Company for research performed with respect to any aspect of the revised Research Plan for which Company has received such grant funds.

(g) If (i) it becomes evident to either party at any time, based on budget forecasts or progress in the Research Plan, that a [**] may [**] for [**], or that the [**], or (ii) the [**] is [**] (each of the foregoing, a "Cost/Timeline Issue"), then either party may, on written notice to the other, call a special meeting of the JSC to address such Cost/Timeline Issue. At such meeting, representatives of each party shall present information in their control with respect to the reasons for such Cost/Timeline Issue, and (if applicable) each party's plan or recommendation for addressing such Cost/Timeline Issue. The JSC shall review and address such Cost/Timeline Issue, and shall determine which of the following actions the parties shall pursue:

(1) develop, approve, and follow an amendment to the Research Plan (such amendment, the "Corrective Plan") to address the Cost/Timeline Issue, which may (subject to the written consent of the affected party in such party's sole discretion) require either party to [**] the DC Research, or provide that the [**] in which case (x) the [**] shall be [**] and/or, if the [**] is more than [**] years after the Second Amendment Effective Date, then the Research Term shall be deemed amended to extend until the earliest of (i) the date upon which the JSC first designates a Development Candidate, (ii) the [**], or (iii) the effective date of any termination of the Research Term pursuant to Second Amendment Section 3, and (y) in addition to their other obligations under the Agreement, the parties shall duly perform their respective obligations pursuant to such Corrective Plan; provided, however, that after the adoption of a Corrective Plan, failure to achieve the [**] or [**] shall not be deemed, by itself, to be a breach of this Agreement, but shall entitle either party to terminate the Research Term pursuant to Second Amendment Section 3;

(2) restructure the Research Plan and related budget in a manner that resolves the Cost/Timeline Issue; provided, however, that such restructuring shall not obligate either party to [**] of the [**] or be deemed to [**]; or

(3) determine that continuation of the DC Research would be futile, in which case the JSC shall recommend to the parties that they terminate the DC Research; provided further, that following such recommendation either party shall have the right to terminate the Research Term pursuant to Second Amendment Section 3.

(h) If the members of the JSC fail to unanimously agree upon one of the three actions described in Second Amendment Section 2(g) (1), (2) and (3), then the matter shall be referred to the parties' Chief Executive Officers, and if the parties' Chief Executive Officers do not agree upon one of such three actions within [**] days after matter referral, then either party shall have the right to terminate the Research Term pursuant to Second Amendment Section 3. If the affected party does not approve the Corrective Plan within [**] days after it is first formally proposed, then either party shall have the right to terminate the Research Term pursuant to Second Amendment Section 3.

3. Special Termination. In addition to the rights to terminate this Agreement as provided in Article 7 of the Agreement, either party shall have the rights to terminate the Research Term as provided in Second Amendment Section 2(g)(1), 2(g)(3) or 2(h) (any such termination of the Research Term, a "Special Termination"). Upon written notice from one party to the other party consistent with the provisions of Second Amendment Section 2(g)(1), 2(g)(3) or 2(h) and specifically identifying the circumstances giving rise to a right of Special Termination, a Special Termination shall go into effect and neither party shall have any rights or obligations with respect to the other party pursuant to this Agreement except as specifically set forth in this Second Amendment Section 3. Upon the effectiveness of a Special Termination:

(a) subject to Second Amendment Section 3(b), Foundation shall automatically have a worldwide, fully-paid up and royalty-free, nonexclusive, nontransferable (except in connection with the assignment of this Agreement pursuant to Section 9.1 of the Agreement), sublicensable (solely as set forth in this Second Amendment Section 3(a)) right (i) under the Company Technology, Licensee Technology, Data, Licensee Data and Company Base IP, to make, have made and import Reversion Candidates and to use Reversion Candidates for its own internal purposes and for pre-clinical research activities ([**] any pre-clinical research performed under good laboratory practice guidelines (such pre-clinical research, "GLP Research")) in the Field, (ii) to access or reference any filings made by Company or its agents with Regulatory Authorities with respect to any Reversion Candidate, (iii) to receive within [**] months of the effectiveness of the Special Termination copies of all Data and Licensee Data and all reports and other information that were (or should have been) accessible to Foundation prior to the Special Termination via the shared electronic collaboration space described in Second Amendment Section 4(a), (iv) to receive within [**] months of the effectiveness of the Special Termination reasonable quantities of existing stock of materials (other than (1) materials that are [**] or (2) materials that [**]) in Company's possession or under its control and (xx) that are specific to, or were used or were contemplated to be used in, the DC Research, and are not commercially available from Third Parties, (yy) that are reasonably necessary for continued research or

preclinical testing of Reversion Candidates or were used in, or were contemplated to be used, in the DC Research, and (zz) the transfer of which would not infringe any Third Party intellectual property rights (and no non-infringing alternative is identified after a reasonable inquiry), trigger a breach of any contractual obligations of Company with respect to a Third Party (other than a Licensee), or [**] trigger any contractual obligation to make payments to a Third Party (other than a Licensee); provided, however, that any subsequent transfers of such materials by Foundation to Third Parties shall be subject to the terms of a materials transfer agreement reasonably acceptable to Company, and (v) to gain access [**] to reasonable quantities of Company's existing stock of Reversion Candidates for its own internal purposes and for pre-clinical research activities ([**] any GLP Research) in the Field; such right to be sublicensable by Foundation to (1) a contract research organization or non-academic Foundation collaborator only upon prior written notice to Company or (2) an academic or governmental Foundation collaborator only with the prior written consent of Company, such consent not to be unreasonably withheld or delayed and only to be withheld based on objective criteria determined by the JSC within [**] months after the Second Amendment Effective Date. At Foundation's request and expense, Company shall provide Foundation with reasonable assistance to facilitate Foundation's practice of the foregoing right, including disclosure of Company Know-How, provision of technical assistance and facilitation of Foundation's efforts to obtain supply of Reversion Candidates from the Third Party who supplied such Reversion Candidate to Company prior to the Special Termination;

(b) each party shall keep the other party reasonably informed with respect to the results of any non-clinical, pre-clinical research ([**] GLP Research) and clinical testing performed upon any Reversion Candidate by or on behalf such party following a Special Termination (which clinical testing and GLP Research, if in the Field, shall only be performed after the obligations set forth in Second Amendment Section 3(c) have been satisfied);

(c) neither party may perform upon, any Reversion Candidate, any clinical testing in the Field or any GLP Research in the Field without first providing written notice to the other party and [**];

(d) if either party provides the other party with notice and [**] pursuant to Section 3(c) of this Second Amendment but the parties do not, within [**] months after such notice, [**] with respect to the [**], then Company may within the next [**] days provide written notice (a "Development Election Notice") to Foundation stating that Company intends to pursue continued Development and commercialization of one or more Reversion Candidates in the Field using Commercially Reasonable Efforts and identifying the Reversion Candidate of greatest interest to Company; such Reversion Candidate shall be deemed to be a Development Candidate selected by the JSC as of the date of the Development Election Notice. If Company provides a Development Election Notice within such [**] day period, then, notwithstanding any other provision of this Second Amendment Section 3, the parties shall have all rights and obligations under this Agreement that apply to periods after the end of the Research Term and the JSC shall resume functioning as specified in Second Amendment Section 5(a). If Company does not provide a Development Election Notice within such [**] day period, then upon Foundation's written notice to Company (such notice, a "Reversion Notice") within the Option Period,

Foundation shall have a Reversionary License and all other rights set forth in Section 6.1(c)(2) of the Agreement;

(e) notwithstanding any other provision of the Agreement to the contrary (except for Second Amendment Section 3(d)), only the provisions of Sections 4.7, 6.1(a), 6.1(b), 6.1(c)(2), 6.1(c)(4), 6.2, 7.5 of the Agreement, and Articles 1, 5, 8, and 9 of the Agreement, Sections 5 and 6 of the First Amendment (*provided*, that the [**] month periods referenced in Sections 5 and 6 of the First Amendment shall terminate [**] months after the effectiveness of the Special Termination), and Sections 1, 3, 4(b)(ii), and 18(a) of this Second Amendment will survive such Special Termination; *provided, however,* that in the event Foundation subsequently obtains a Reversionary License pursuant to Second Amendment Section 3(d), then all provisions of this Agreement will continue to apply except to the extent terminated pursuant to Section 6.1(c)(2)(vi) of the Agreement; and

(f) except as explicitly set forth in Second Amendment Section 3(a) with respect to certain optional costs payable by Foundation, Foundation shall not have any obligations to pay for any research-associated costs incurred after the effective date of the Special Termination.

4. Research Reports and Access to Information. In lieu of the Research Reports and other information and communications that would otherwise be due from Company under Sections 2.4 and 2.7 of the Agreement during the Research Term, or pursuant to Section 3 of the First Amendment, Company shall make the following reports and information available:

(a) Information. Promptly after the Second Amendment Effective Date, Company will establish a shared electronic collaboration space that enables designated representatives of Foundation to access and provide information on the progress of the DC Research. For clarity, the persons listed on Exhibit SA-5 of this Second Amendment are, as of the Second Amendment Effective Date, designated representatives of Foundation for such purpose. Foundation may remove any such designated representative at any time upon written notice to Company. Foundation may also appoint new designated representatives subject to the conditions specified in Second Amendment Section 18(f). Such information shall include agendas and minutes of team meetings, presentations, correspondence between the parties, and data and reports from the DC Research, as well as monthly FTE reports (which reports shall be posted no later than [**] days after the end of the applicable month and shall list the number of hours that each person (identified by name and general job description (e.g., "chemist")) worked on the DC Research during such month). Company shall post data from the ongoing conduct of the DC Research to such electronic collaboration space on a regular and continuing basis; provided, that (i) the frequency of such posting may be adjusted by consent of the JSC, and (ii) in the absence of any such consent, Company shall post such data at the same time and in the same format as made available to Company's internal project leadership team (a sample of which format is appended as Exhibit SA-2). Company shall have the right to limit access to sensitive data (by way of example, but not limited to, non-public chemical structures) to a mutually-agreeable list of representatives of Foundation. Such list, as of the Second Amendment Effective Date, is set forth on Exhibit SA-6. Foundation may remove any such representative from such list at any time upon written notice to Company. Foundation may also add new representatives to such list subject to the conditions specified in Second Amendment Section 18(f).

(b) Reports. (i) Within [**] days of the end of each [**] or at least [**] prior to any [**] meeting of the JSC (whichever comes first), or such other regular times as the parties may otherwise agree, Company shall provide to Foundation with a reasonably detailed written summary report of the results (including Company's analysis thereof) and progress of the DC Research during such [**] and expectations for DC Research to be conducted during the immediately subsequent [**], and (ii) within [**] days of completion of the DC Research or termination of the DC Research on account of a Special Termination or pursuant to Article 7 of the Agreement, Company shall provide to Foundation a final report summarizing the status and accomplishments of the DC Research and containing the recommendations by Company with respect to selection of a Development Candidate with respect to one Research Project and for further research towards a potential Development Candidate

with respect to the other Research Project. Company will promptly provide all information reasonably requested by Foundation regarding the DC Research described in any report provided pursuant to this Section 4(b) of this Second Amendment.

(c) Availability for Communications. In addition to the foregoing and to Company's obligations under Section 2.5 of the Agreement, Company will make appropriate representatives of the scientific team conducting the DC Research available for conference calls and meetings with appropriate representatives of Foundation at reasonable times and places for informal discussion of the progress of the DC Research. In further addition, the Foundation may, at its option, during the Term, schedule up to [**] formal program review meetings with Company personnel and those of Foundation's Third Party advisors who (i) have been designated by Foundation in compliance with Second Amendment Section 18(f), and (ii) are reasonably acceptable to Company. Such meetings will be held at the times and locations mutually agreed upon by the parties. The purpose of such meetings will be to review the progress of the Research relative to the Research Plan.

5. Governance. The parties agree to the following provisions with respect to governance of their collaboration:

(a) Joint Steering Committee. The parties will establish a joint steering committee ("JSC") consisting of equal representation from Foundation and Company within [**] days after the Second Amendment Effective Date. The parties acknowledge and agree that the individuals listed on Exhibit SA-8 have been approved, as of the Second Amendment Effective Date, to serve as the Foundation's representatives to the JSC and there is no need for the parties to perform the procedures set forth in Second Amendment Section 18(f) with respect to their appointment to the JSC. The JSC shall be comprised of at least [**] representatives of each party, each with appropriate decision-making authority to enable the JSC to fulfill its obligations under this Agreement, and which in the case of Foundation may be Third Party advisors of Foundation, provided they are appointed pursuant to the conditions specified in Second Amendment Section 18(f). Changes in the designation of JSC members by each party may occur at any time during the Term upon written notification by a party to the other party. The JSC, as its first order of business, shall select a chairperson from one party and a secretary from the other party, to alternate on an annual basis. Subject to the confidentiality provisions of the Agreement and any appropriate agreements with respect to intellectual property or conflicts of interest, the JSC may invite other representatives of the parties with special skills or knowledge (and who, in

the case of Foundation, may be Third Party advisors of Foundation) to attend JSC meetings where appropriate. Each party shall disclose to the other its proposed agenda items in advance of each JSC meeting, and the chairperson shall distribute a draft agenda reflecting such proposed agenda items reasonably in advance of each meeting. The JSC shall adopt such other procedural rules as are necessary or convenient for its work. Each party shall be responsible for all travel and other costs for its representatives to attend meetings of, and otherwise participate on, the JSC. The JSC shall continue to function until the earliest of: (i) the effective date of a Special Termination, (ii) the Company's receipt of a Reversion Notice or a Buy-Out Notice or (iii) the end of the Term. If the JSC stopped functioning on account of a Special Termination and the Company subsequently provides a Development Election Notice pursuant to Second Amendment Section 3(d), then the JSC shall resume functioning promptly upon the Foundation's receipt of such Development Election Notice. Such reconvened JSC shall have the duties specified in Second Amendment Section 2(c) and, regardless of whether Proof-of-Concept has been achieved as of the date of the Development Election Notice, it shall meet and make decisions in accordance with the provisions of Second Amendment Section 5(e) (and not Second Amendment Section 5(d)).

(b) Duties of the JSC during the Research Term. During the Research Term, the JSC shall be responsible for:

(i) monitoring the parties' activities under the Research Plan and the Agreement;

(ii) reviewing and approving amendments to the Research Plan (and related budget), and at least once each calendar year formally reviewing and updating the Research Plan (and related budget) on a comprehensive basis;

(iii) in connection with the review and approval of the Research Plan (and related budget) and any amendments thereto, identifying appropriate resources necessary to conduct the DC Research and adjusting, as necessary to further the purpose of the DC Research, the budget for the Research Plan;

(iv) establishing timelines and criteria for continuation/discontinuation decision points under the DC Research;

(v) establishing and revising minimum activity and safety criteria for Lead Candidates from each Research Project within the DC Research (which criteria may be different for the [**] Research Projects within the DC Research);

(vi) maintaining and updating at each JSC meeting during the Research Term, one list for each of the [**] Research Projects within the DC Research that identifies and rank orders all potential and actual Lead Candidates and Development Candidates from such Research Project and denotes all Development Candidates and between [**] and [**] potential or actual Lead Candidates from such Research Project as "Reversion Candidates";

(vii) deciding whether to pursue (1), (2) or (3) of Section 2(g) of this Second Amendment in the event of a Cost/Timeline Issue;

(viii) establishing criteria for, and designating, Development Candidate(s);

(ix) providing a forum for discussion/presentation regarding, and serving as the sole governance body for decision-making regarding, research, Development, commercialization, and Collaboration Activities with respect to Drug Candidates, Reversion Candidates, Development Candidate(s) and Product(s); for clarity the JSC's role as such sole governance body shall not prevent the Company or its Licensee from making decisions necessary or useful to implement decisions made by the JSC regarding research, Development, commercialization, and Collaboration Activities with respect to Drug Candidates, Reversion Candidates, Development Candidate(s) and Product(s), so long as such implementation decisions are consistent with and faithful to the intent of the JSC's decision;

(x) prior to the designation of a Development Candidate, preparing the Development Plan for such Development Candidate and reviewing and updating the Development Deadline Document as it may deem advisable, in each case as further provided in Article 3 of the Agreement;

(xi) serving in the role specified in Second Amendment Section 10 with respect to transactions arising in connection with Collaboration Activities;

(xii) establishing policies and procedures governing scientific publications and presentations, and if the JSC deems it advisable, establishing a publication committee to administer such policies and procedures, as further provided in Section 5.4(a) of the Agreement;

(xiii) except for those rights and obligations specified in Section 4 of this Second Amendment, serving in lieu of the parties with respect any rights or obligations to review, communicate, inform, meet or discuss otherwise provided for in Sections 2.2, 2.4, and 2.7 of the Agreement;

(xiv) developing the criteria specified in Second Amendment Sections 3(a) and 18(f) within [**] months of the Second Amendment Effective Date;

(xv) reviewing scientific and medical literature to identify diseases, indications or medical conditions that, [**] or [**], are [**] for [**] and [**] diseases, indications or medical conditions [**];

(xvi) performing those other tasks specifically allocated to it in this Agreement that are applicable during the Research Term; and

(xvii) otherwise serving as a forum for exchanging information and discussing the progress of the collaboration between Company and Foundation pursuant to the Agreement.

(c) Duties of the JSC Following the Research Term. Following the Research Term, the JSC shall be responsible for:

(i) at the first JSC meeting after the end of the Research Term, (1) reviewing each potential or actual Lead Candidate that was not designated as a Development Candidate

during the Research Term and either designating it as a Development Candidate or determining that it does not meet the criteria for designation as a Development Candidate and (2) preparing a final list (which can only be subsequently changed by the written agreement of the parties) for each of the [**] Research Projects within the DC Research that identifies and rank orders all potential and actual Lead Candidates and Development Candidates from such Research Project and denotes all Development Candidates and between [**] and [**] potential or actual Lead Candidates from such Research Project as "Reversion Candidates";

(ii) following the designation of a Development Candidate, and at least [**] thereafter, conducting a formal review and comprehensive update of the Development Plan and Development Deadline Document for such Development Candidate, in each case as further provided in Article 3 of the Agreement;

(iii) monitoring Company's and its Affiliates and Licensees activities with respect to the Development Plan and Development Deadline Document;

(iv) providing a forum for discussion/presentation regarding, and serving as the sole governance body for decision-making regarding, Development, commercialization, and Collaboration Activities with respect to Reversion Candidates, Development Candidate(s) and Product(s); for clarity the JSC's role as such sole governance body shall not prevent the Company or its Licensee from making decisions necessary or useful to implement decisions made by the JSC regarding Development, commercialization, and Collaboration Activities with respect to Reversion Candidates, Development Candidate(s) and Product(s), so long as such implementation decisions are consistent with and faithful to the intent of the JSC's decision;

(v) serving in the role specified in Second Amendment Section 10 with respect to transactions arising in connection with Collaboration Activities;

(vi) establishing policies and procedures governing scientific publications and presentations, and if the JSC deems it advisable, establishing a publication committee to administer such policies and procedures, as further provided in Section 5.4(a) of the Agreement;

(vii) except for those rights and obligations specified in Section 4 of this Second Amendment, serving in lieu of the parties with respect any rights or obligations to review, communicate, inform, meet or discuss otherwise provided for in Sections 2.2, 2.4, and 2.7 of the Agreement;

(viii) reviewing scientific and medical literature to identify diseases, indications or medical conditions that, [**] or [**], are [**] for [**] and [**] diseases, indications or medical conditions [**];

(ix) performing those other tasks specifically allocated to it in this Agreement that are applicable after the Research Term; and

(x) otherwise serving as a forum for exchanging information and discussing the progress of the collaboration between Company and Foundation pursuant to the Agreement.

(d) Meetings and Decision-Making by the JSC — Before Proof-of-Concept. During the Research Term and through achievement of Proof-of-Concept, the JSC shall meet periodically as needed, but in no event less than [**], in person (with locations to alternate between the parties) or by teleconference or other electronic means as mutually agreed, to discuss matters within its jurisdiction. In addition, the JSC may agree to hold special meetings at any time on reasonable notice given by the chairperson or the secretary to the other members of the JSC. Unless waived by a party in writing, at least [**] JSC representatives of each party must participate in a meeting of the JSC in order for there to be a quorum at such meeting. The members of the JSC shall seek to make all determinations to be made by them unanimously following full discussion thereof (with each party's representatives having, collectively, one (1) vote). If the JSC is unable to reach a unanimous decision on any matter within its jurisdiction, the parties' respective Chief Executive Officers shall meet in person to attempt to resolve the matter in good faith. If the parties' respective Chief Executive Officers are unable to reach agreement on a matter referred to them pursuant to the foregoing sentence within [**] days after the matter referral, then either party may by written notice to the other submit the matter to Baseball Arbitration as provided in Section 17 of this Second Amendment; provided, however, that the following matters shall not be subject to such referral to Baseball Arbitration, and any disputes arising in the JSC with respect to them may only be resolved by mutual agreement of the parties: (i) [**]; (ii) any [**] described in Second Amendment Section [**]; (iii) any changes to the [**] that would require [**] than contemplated in the [**]; and (iv) deciding whether to pursue ([**] of this Second Amendment in the event of a [**].

(e) Meetings and Decision-Making by the JSC — Following Proof-of-Concept. Following achievement of Proof-of-Concept, the JSC shall meet periodically as needed, but in no event less than [**] during each calendar year, in person (with locations to alternate between the parties) or by teleconference or other electronic means as mutually agreed, to discuss matters within its jurisdiction. In addition, the JSC may agree to hold special meetings at any time on reasonable notice given by the chairperson or secretary to the other members of the JSC. Unless waived by a party in writing, at least [**] JSC representatives of each party must participate in a meeting of the JSC in order for there to be a quorum at such meeting. The members of the JSC shall seek to make all determinations to be made by them unanimously following full discussion thereof (with each party's representatives having, collectively, one (1) vote). If the JSC is unable to reach a unanimous decision on any matter within its jurisdiction, the parties' respective Chief Executive Officers shall attempt to resolve the matter in good faith. If the parties' respective Chief Executive Officers are unable to reach agreement on a matter referred to them pursuant to the foregoing sentence within [**] days after the matter referral, then [**] shall have the deciding vote on the matter; provided, however, that the following matters shall not be subject to such [**] final determination, and any disputes arising in the JSC with respect to them may only be resolved as set forth below: (i) any [**], and (ii) the [**] with respect to [**] in connection with [**] set forth in, and subject to, Second Amendment Section [**].

(f) Meeting Minutes. The secretary (or if absent, such acting secretary as the chairperson shall designate) shall be responsible for preparing the minutes of the JSC meeting. Such JSC meeting minutes shall provide a description in reasonable

detail of the discussions held at the meeting, and a list of any actions, decisions or determinations made by the JSC. Unless otherwise agreed by the JSC, the secretary shall distribute draft minutes of each meeting within

[**] days after the meeting for review and comment, and final minutes shall be approved by both parties within [**] days after the meeting.

(g) Joint Teams. Within [**] days after the Second Amendment Effective Date, the JSC shall establish a Joint Team with appropriate representation from the parties, which in the case of the Foundation may be Third Party advisors of Foundation appointed pursuant to the conditions specified in Second Amendment Section 18(f), to assist the JSC in the execution of the Research Plan. The parties acknowledge and agree that the individuals listed on Exhibit SA-8 have been approved, as of the Second Amendment Effective Date, to serve as the Foundation's representative to the Joint Team and there is no need for the parties to perform the procedures set forth in Second Amendment Section 18(f) with respect to their appointment to the Joint Team. The JSC shall have the authority to establish one or more additional Joint Teams with appropriate representation from the parties to assist the JSC in the performance of its duties. The JSC may establish such procedural rules and meeting schedules for such Joint Teams as it deems appropriate; provided, that unless otherwise agreed by the JSC each Joint Team shall meet at least [**], and shall report on its activities to the JSC at regularly-scheduled JSC [**] meetings. The JSC may change the composition of any Joint Team at any time upon notice to the parties.

(h) Appointment of JSC Members and Joint Team Members. The appointment of members of the JSC and any Joint Team is a right of each party and not an obligation and shall not be a "deliverable" as defined in EITF Issue No. 00-21. Each party shall be free to determine not to appoint members to the JSC and any Joint Team, and at any time during the Term and for any reason, either party shall have the right to withdraw from participation in the JSC and any Joint Team upon written notice to the other party, which notice shall be effective immediately upon receipt. If a party ("Appointing Party") does not appoint members of the JSC or any Joint Team, or withdraws from the JSC or any Joint Team, it shall not be a breach of this Agreement, nor shall there be any associated penalty due nor shall there be any impact on the consideration otherwise provided for or due to the Appointing Party under this Agreement, and unless and until such persons are again appointed: (i) the other party, without regard to the provisions of this Second Amendment Section 5 with respect to voting, quorum or dispute resolution, may discharge the roles of the JSC and any Joint Team for which appointments were not made or with respect to which a withdrawal or removal has occurred by the Appointing Party (including designating a chairperson and secretary of the JSC and making all decisions within the decision-making authority of the JSC, which decisions shall be binding thereafter on both parties) and (ii) where the Appointing Party has not made appointments to the JSC or has withdrawn from the JSC, the Appointing Party shall not participate in any meetings of the JSC and shall not have the right to approve the minutes of any JSC meeting. If, at any time following the Second Amendment Effective Date, a party has not appointed or has pursuant to this Second Amendment Section 5(h) withdrawn from the JSC or any Joint Team, and such party wishes to resume participating in the JSC or any Joint Team, such party shall notify the other party in writing and, thereafter, such notifying party's designees shall be entitled to attend any subsequent meeting of the JSC or any Joint Team and to participate in the activities of, and decision-making by, the JSC or any Joint Team, in each case as provided in this Second Amendment Section 5 as if a failure to appoint or submitting the withdrawal notice had not occurred.

6. Information Concerning other SMA Efforts. The parties acknowledge that a goal of Foundation in funding the DC Research is to identify and advance the compound most likely to advance rapidly to human clinical trials directed towards the treatment, mitigation or prevention of SMA, and that therefore the parties may have an interest in negotiating funding of other research and development efforts conducted by Company instead of, or in addition to, the Research Projects. In furtherance of this objective, Company will make available to Foundation on a confidential basis regular reports with respect to progress and summary data with respect to Company's other internal efforts directed towards the approval of a compound for the treatment, mitigation or prevention of SMA. In addition, Company will make available to Foundation general product profiles showing, on a comparative basis, the status of potential Development Candidates from the DC Research against other potential therapeutic agents being pursued by Company in the treatment, mitigation or prevention of SMA (whether internal or in collaboration with Third Parties) in the format provided in Exhibit SA-3 to this Second Amendment; provided, however, that such obligation shall not require Company to breach any condition of any agreement in effect as of the Second Amendment Effective Date. Company will use Commercially Reasonable Efforts to ensure that it is able to share the information specified in this Second Amendment Section 6 with respect to any Third Party agreements entered in to following the Second Amendment Effective Date, and may only enter into such Third Party Agreements if it notifies Foundation reasonably in advance of entering in to such agreements to allow further discussions and potential negotiations with such Third Party with respect to such sharing of information. If, based on information made available pursuant to this Second Amendment Section 6, either party is of the opinion that a change in funding or approach may be advisable, then such party may propose to the JSC, and the JSC shall conduct, an evaluation of the merits of such proposed change that includes a report and recommendation thereon to the parties.

7. Foundation Access to Company Meetings Following Declaration of a Development Candidate. Following the JSC's determination that a particular compound is a Development Candidate and through the earlier of Regulatory Approval, abandonment of Development of such Development Candidate, or the granting of a Reversionary License to Foundation, Company shall invite a representative of Foundation (to be designated by Foundation) to observe regularly scheduled monthly meetings of the Company team charged with Development of such Development Candidate, subject to the terms of Second Amendment Section 18(f); provided, however, that failure of Foundation to designate such representative or failure of such representative to attend such meetings shall not constitute a breach of this Agreement. Company may request that Foundation representative recuse themselves from such meetings (or portions of such meetings) (a) that do not relate specifically to a Development Candidate, (b) to prevent the breach of an applicable legal or regulatory obligation of confidentiality or privacy or avoid a conflict of interest, (c) to protect the attorney-client privilege, and/or (d) to preserve intellectual property rights.

8. Development of Products.

(a) Article 3 of the Agreement (captioned "Development of Products") shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"**3. DEVELOPMENT OF PRODUCTS.**

"3.1 Development Plan and Development Deadline Document. Upon selection of a Development Candidate, the JSC will meet to prepare a plan for the Development of such Development Candidate (such plan, the "Development Plan" for such Development Candidate") and to conduct a formal review of and prepare a comprehensive update to Exhibit SA-4A to the Second Amendment (the "Development Deadline Document") that reflects anticipated activities directed towards Development and commercialization of such Development Candidate through Regulatory Approval in the United States, in each case taking into consideration available information concerning such Development Candidate, the interests of SMA patients, the intellectual property and regulatory landscape and the commercial potential of the Development Candidate. The parties acknowledge and agree that Exhibit SA-4A takes into account many delays in Development and receipt of Regulatory Approval that, while possible, are not anticipated as of the Second Amendment Effective Date to be likely; Company's expectations, as of the Second Amendment Effective Date, of the activities required to obtain Regulatory Approval and its goal timelines for completing such activities are set forth in Exhibit SA-4B. When preparing the Development Plan and updating the Development Deadline Document for each Development Candidate, the JSC shall consider whether to obtain, (a) "Orphan Product" designation from the FDA, and (b) research funding from the FDA's Office of Rare Diseases or other government agencies to support human clinical trials conducted for such Development Candidate, in each case taking into consideration the protection of intellectual property rights and confidential information. The Development Plan shall set forth, in at least the level of detail included in the Company's or its Licensee's plans for developing other preclinical or clinical (whichever reflects the status of the Development Candidate at such time) pharmaceutical products, both major and minor Development activities planned to be conducted with respect to such Development Candidate by or on behalf of Company or its Affiliates or Licensees, the anticipated timeline for performing such activities, the goals of such activities and the anticipated timeline for achieving such goals. The Development Deadlines Document shall set forth the deadline by which each major Development activity must be performed by on behalf of Company or its Affiliates or Licensees if the Company wishes to avoid granting the Foundation the right to obtain a Reversionary License pursuant to Section 3.3 of the Agreement. No change can be made to any Development Plan or Development Deadline Document without the approval of the JSC unless such change is approved by the parties' respective Chief Executive Officers pursuant to Second Amendment Section 5(d) or 5(e), is implemented by Baseball Arbitration

in accordance with Second Amendment Sections 5(d) and 17, or is approved by the Foundation in accordance with Second Amendment Section 9(b)(1).

"3.2 Diligence. Prior to selection of a Development Candidate, Company shall (i) perform the activities set forth in the Research Plan in a timely and complete manner, (ii) use Commercially Reasonable Efforts to achieve the goals of the DC Research within the time and budget allotted therefor in the Research Plan, and (iii) also have the research and Development obligations set forth in Section 2.5 of the Agreement. Following selection of a Development Candidate, Company shall use Commercially Reasonable Efforts to Develop and commercialize (whether directly, through an Affiliate, or in collaboration with one or more Third Parties, through licensing or some combination of the foregoing, all in compliance with the other applicable terms of this Agreement), for the treatment, mitigation or prevention of SMA or any other disease, indication or medical condition approved in writing by Foundation, at least one Product from such Development Candidate.

In the event that the Development of a Development Candidate [**] toxicology studies governed by good laboratory practices ("GLP Toxicology Studies") that causes Company to [**] that [**] is [**], then Company shall promptly notify Foundation in writing and Company shall spend up to [**] dollars ($[**]) Developing a Reversion Candidate through the start of GLP Toxicology Studies, provided that such Development does not [**] to [**] that such [**]. Upon the initiation of GLP Toxicology Studies for such Reversion Candidate, it shall be deemed a Development Candidate and Company shall have the diligence obligations set forth in the second sentence of this Section 3.2 of the Agreement. In the event that the Development of a Development Candidate [**] of [**] that [**] to [**] that such [**], then Company shall promptly notify Foundation in writing and Company shall, within [**] days of such notice, notify Foundation that Company has decided to do one of the following: (a) Develop one or more potential or actual Reversion Candidates or Lead Candidates at its own expense and in accordance with the terms and conditions of this Agreement, (b) Develop one or more potential or actual Reversion Candidates or Lead Candidates if Foundation is willing to pay for [**] percent ([**]%) of the costs of such Development for a [**] month period while the parties negotiate in good faith a separate agreement governing the further Development of such potential or actual Reversion Candidates or Lead Candidate(s); *provided*, that in the event the parties are unable to reach such separate agreement following good faith negotiations, then Company shall have [**] days following the end of such [**] month period to notify the Foundation of its decision to elect either option (a) or (c), or (c) stop all Development work on potential and actual Reversion Candidates or Lead Candidates and Development Candidates. If Company chooses option (a), then Company shall use Commercially Reasonable Efforts to Develop such Reversion Candidates and/or Lead Candidates through the start of GLP Toxicology Studies; upon the initiation of GLP Toxicology Studies for any such Reversion Candidate or Lead Candidate, it shall be deemed a Development Candidate and Company shall have the diligence obligations set forth in the second sentence of this Section 3.2 of the

Agreement. If Company chooses option (c), then upon written notice (a "Reversion Notice") to Company, Foundation shall have the Reversionary License and other rights set forth in Section 6.1(c)(2) of the Agreement.

"3.3 Development Benchmarks. Following designation of a Development Candidate, and in addition to Company's general diligence obligation set forth in Section 3.2 of the Agreement, Company shall use Commercially Reasonable Efforts to perform the activities set forth in the Development Plan in accordance with the timeline specified therein and to complete each activity set forth in the Development Deadline Document prior to the applicable deadline specified therein, as such Development Plan or Development Deadline Document may be amended consistent with the terms of this Agreement, with respect to Development of a Product based on such Development Candidate; provided, however, that:

"(a) the JSC shall conduct a formal review of and comprehensive update to such Development Plan and Development Deadline Document on an annual basis to reflect, on a good faith basis, information from the DC Research, ongoing clinical or supportive non-clinical trials, or other factors that may impact the activities, timelines, milestones and goals set forth such Development Plan or the deadlines set forth in such Development Deadline Document;

"(b) a failure of Company, despite Commercially Reasonable Efforts, to meet any deadline set forth in a particular Development Deadline Document (as amended by the JSC) with respect to the relevant Development Candidate (each, a "Benchmark Trigger") shall not create a breach of the Agreement, but shall instead trigger the availability of a right on the part of Foundation to obtain a Reversionary License in accordance with the following terms:

"(i) if Foundation believes a Benchmark Trigger has occurred, it shall provide written notice to Company setting forth in reasonable detail those aspects of the Development Deadline Document that have created such Benchmark Trigger.

"(ii) Company shall have [**] days to respond to a notice of Benchmark Trigger, which response shall either be (1) to cure the Benchmark Trigger (if it is capable of being cured), or (2) to propose a corrective plan to address the Benchmark Trigger, which shall take the form of a proposed amendment to the Development Deadline Document.

"(iii) if Company proposes a corrective plan to address the Benchmark Trigger, Foundation shall have [**] days to accept or reject such corrective plan. The parties may extend such [**] day period by mutual consent to engage in good faith negotiations

directed towards arriving at a mutually-agreeable form of such corrective plan with respect to such Benchmark Trigger.

"(iv) if Company fails to respond to a Benchmark Trigger notice or cure the applicable Benchmark Trigger within [**] days of such Benchmark Trigger Notice, or, following the acceptance of a corrective plan for a Benchmark Trigger by Foundation fails to use Commercially Reasonable Efforts to execute such corrective plan, or following a PTC Corporate Change the entity primarily responsible for Company's obligations under this Agreement fails to provide an M&A Certification as more fully set forth in Second Amendment Section 9(b)(4), then immediately as of such occurrence Foundation shall have the right to obtain the Reversionary License and other rights set forth in Section 6.1(c)(2) of the Agreement (a "Buy-Out Right").

"(v) Foundation may exercise its Buy-Out Right by providing written notice (a "Buy-Out Notice") to Company and the first installment payment described in Section 6.1(c)(3)(iii)(A) of the Agreement, such Buy-Out Notice to be effective upon the occurrence of both (A) receipt by Company and (B) availability of funds with respect to such first installment payment (provided that such funds shall be deemed to be available on the [**] business day after the Company's receipt of such initial payment if the Company does not deposit such payment within [**] after such receipt). Upon the effectiveness of such Buy-Out Notice, the terms of Section 6.1(c)(2) of the Agreement shall apply.

"(vi) notwithstanding the foregoing, if Foundation fails to exercise a Buy-Out Right within [**] years of the date of the accrual of such Buy-Out Right, and other than with respect to the circumstances giving rise to such Buy-Out Right Company is in compliance with the terms of this Agreement, then such Buy-Out Right shall lapse and no longer be exercisable by Foundation. For clarity, the foregoing operates on a Buy-Out Right by Buy-Out Right basis, with Foundation having a full [**] year period to exercise each Buy-Out Right.

"3.4 Decisions to Discontinue Development or Commercialization.

(a) At the request of Company, the JSC shall determine, based on a comparison of test data for a particular Development Candidate (both alone and in combination with another treatment) and for the applicable Available Product and through the application of objective criteria previously established by the JSC (or by a mutually agreed independent technical expert if the JSC is not able to agree upon such objective criteria within [**] days after either party provides written notice to the other that

it intends to arrange for a technical expert to decide such criteria), which criteria shall include without limitation [**] and [**], whether such Development Candidate (both alone and in combination with another treatment) appears to be less desirable as a therapeutic option in the Field than such Available Product. If, following such a determination, Company informs Foundation in writing that it intends to cease further Development and commercialization of the applicable Development Candidate, then Foundation shall have: (i) upon Foundation's written notice to Company (such notice, a "Reversion Notice"), a Reversionary License and all other rights set forth in Section 6.1(c)(2) of the Agreement if such Available Product [**] (a "[**]"), or (ii) a Buy-Out Right if such Available Product is a [**], such right to be exercisable by Foundation on the terms provided in Sections 3.3(b)(v) and (vi) of the Agreement.

(b) Company shall notify Foundation in writing if Company has, in its good faith judgment, decided that a particular Development Candidate is not commercially viable, which decision shall not be based, in whole or in part, upon the size of the addressable patient population for such Development Candidate. Such notice shall include a written explanation of the basis for Company's decision. Unless the parties enter into a separate agreement pursuant to which [**] the Development or commercialization of such Development Candidate, Foundation shall, upon written notice to Company (such notice, a "Reversion Notice"), have a Reversionary License and all other rights set forth in Section 6.1(c)(2) of the Agreement.

(c) Company shall notify Foundation in writing if Company has, in its good faith judgment, determined that (i) based on advice of outside patent counsel, the pharmaceutical preparation, composition of matter, method of manufacture or method of use of a particular Development Candidate is covered by at least one issued and apparently valid and enforceable United States Patent of a Third Party and (ii) it is not possible for Company (or its Affiliate or Licensee, as applicable) to obtain a license under such Third Party Patents on commercially reasonable terms (a "Third Party Patent License"). Upon receipt of such notice from Company and provision by Foundation of a written notice to Company (such notice, a "Reversion Notice"), Foundation shall have a Reversionary License and all other rights set forth in Section 6.1(c)(2) of the Agreement.

(d) Company shall notify Foundation in writing if Company has, in its good faith judgment, decided to cease all Development and commercialization of a particular Development Candidate and it believes that such cessation is not a breach of the obligations set forth in Section 3.2 of the Agreement. Such notice shall include a written explanation of the basis for Company's belief. Unless the Foundation notifies Company in writing that it does not agree with such belief and that Company is

obligated to continue Development and commercialization of such Development Candidate in accordance with Sections 3.2 and 3.3 of the Agreement, then upon Foundation's written notice to Company (such notice, a "Reversion Notice"), Foundation shall have a Reversionary License and all other rights set forth in Section 6.1(c)(2) of the Agreement."

(b) The parties acknowledge their continued interest in Research Project B in the area of [**] which is [**], and their good faith intention to continue negotiations (including negotiations with any Third Parties) with respect to finding a way to fund and advance research directed towards Research Project B. Therefore, notwithstanding anything to the contrary in this Second Amendment, the amendments effectuated by this Second Amendment shall not apply to such Research Project B or any Lead Candidates identified during the course of such Research Project B. Instead, the terms of the Agreement (including those amendments implemented pursuant to the First Amendment) as they existed prior to amendment by this Second Amendment shall continue to apply, after the Second Amendment Effective Date, exclusively to such Research Project B and any Lead Candidates identified during the course of such Research Project B.

9. PTC Corporate Change:

(a) M&A Approval Request. Company shall have the option to notify a designated representative of Foundation in writing (an "M&A Approval Request") no later than [**] days prior to the entry into a definitive written agreement involving a PTC Corporate Change. Such M&A Approval Request shall include the identity of the proposed acquiring or merging entity or entities, the expected relationship (if any) between Company and its Affiliates, Company's shareholders, and Company's management following such PTC Corporate Change, and the expected impact of such PTC Corporate Change on Company's obligations under the Agreement. In addition, subject to appropriate confidentiality protections and the consent of the potential acquiring or merging entity or entities (to the extent such information is information of the potential acquiring or merging entity or entities or relates to the economics or financial terms of the potential PTC Corporate Change), Company shall promptly provide to Foundation's designated representative any supplemental information concerning such potential PTC Corporate Change as Foundation shall reasonably request. If Foundation responds to such M&A Approval Request by consenting to such proposed PTC Corporate Change prior to Company's entry into a definitive written agreement involving a PTC Corporate Change, then the terms and conditions of this Agreement shall remain in full force and effect without alteration, and Foundation shall sign such documents and provide such consents as may be reasonably required to effectuate the proposed PTC Corporate Change as described in such M&A Approval Request. If Foundation does not respond prior to Company's entry into a definitive written agreement involving a PTC Corporate Change, or responds by denying consent prior to Company's entry into a definitive written agreement for such PTC Corporate Change, then the consequences in Second Amendment Section 9(b) shall apply. Having given an M&A Approval Request to Foundation, Company shall not enter into a definitive written agreement involving a PTC Corporate Change contemplated in such M&A Approval Request prior to the earlier of (i) receipt

of a response from Foundation as specified in this Second Amendment Section 9(a), or (ii) [**] days following the provision of such M&A Approval Request to Foundation.

(b) Parallel Notification Option. If with respect to a particular definitive written agreement that would result in the a PTC Corporate Change, Company has not given the M&A Approval Request provided for in Second Amendment Section 9(a), or

if Company has given such M&A Approval Request and Foundation has either failed to respond within [**] days or responded by denying consent, then within [**] of entering into such definitive written agreement, Company shall provide written notice thereof to a designated representative of Foundation (the "M&A Notice"). Such M&A Notice shall include the identity of the proposed acquiring or merging entity or entities, the expected relationship (if any) between Company and its Affiliates, Company's shareholders, and Company's management following such PTC Corporate Change, and the expected impact of such PTC Corporate Change on Company's obligations under the Agreement. Unless otherwise agreed by Foundation, (i) in connection with an M&A Notice given by Company or (ii) if Foundation does not respond to, or denies, an M&A Approval Request pursuant to Second Amendment Section 9(a), then the following terms and conditions shall apply effective upon Company's entry into such definitive written agreement:

(1) Notwithstanding the provisions of Second Amendment Sections 5(b)(x), 5(c)(ii), 5(d) and 5(e), any updates or amendments to the Development Plan (including the initial preparation thereof; provided, however, if as of Company's entry into such definitive written agreement a DC has been selected, but no Development Plan exists, any failure of the JSC to agree on preparation of an initial Development Plan shall be escalated to the Chief Executive Officers and, if required, referred to Baseball Arbitration as provided in Second Amendment Section 5(d)) or Development Deadline Document shall require the prior approval of Foundation in its sole discretion.

(2) Notwithstanding the provisions of Second Amendment Section 5(e), if following the achievement of Proof-of-Concept the JSC is unable to reach a unanimous decision on any matter within its jurisdiction (other than an update or amendment to the Development Plan or Development Deadline Document), and the parties' respective Chief Executive Officers are not able to resolve the matter in good faith as set forth in Second Amendment Section 5(e), then either party may by written notice to the other submit the matter to Baseball Arbitration as provided in Section 17 of this Second Amendment;

(3) If this Agreement is not assigned upon the consummation of such PTC Corporate Change to the entity that gained control of Company or its assets as a result of such PTC Corporate Change, then such entity shall enter into a written agreement with Foundation wherein such entity shall guarantee the performance of Company's obligations pursuant to this Agreement; and

(4) The entity that, following the PTC Corporate Change, will be principally responsible for the obligations of Company under the Agreement shall have [**] days following the consummation of such PTC Corporate Change to (A) have a member of the executive management team of such entity who is responsible for Development of products being developed by Company prior to the PTC Corporate Change participate in a meeting with representatives of Foundation at the Foundation's headquarters to discuss

such entity's plans for conducting the DC Research (if not completed prior to the PTC Corporate Change) and for Developing Products based on Development Candidates and (B) provide to Foundation a written certification (the "M&A Certification") by an authorized officer of such entity (i) affirming such entity's intention to perform the Company's obligations under the Agreement, (ii) summarizing in reasonable detail such entity's plans with respect to conduct of any part of the DC Research not performed by Company as of the effectiveness of the PTC Corporate Change, including a demonstration that sufficient funds, FTEs and other resources have been allocated to the performance of such DC Research, and (iii) summarizing in reasonable detail such entity's plans with respect to execution of the Development Plan and completion of the activities set forth in the Development Deadline Document prior to the deadlines specified therein, including a demonstration that sufficient funds, FTEs and other resources have been allocated to the performance of such Development, and such entity's business plans for the Development Candidates; *provided*, that failure to provide such M&A Certification in the time frame specified in this Second Amendment Section 9(b)(4) shall entitle Foundation to exercise the Buy-Out Right specified in Section 3.3(b)(iv) of the Agreement, such right to be exercisable by Foundation on the terms provided in Sections 3.3(b)(v) and (vi) of the Agreement.

(c) Special Provisions in Connection with M&A Approval Request and/or M&A Notice. The parties recognize the special sensitivity of the information contained in an M&A Approval Request and/or and M&A Notice, and agree that any such notice and its contents are Confidential Information of Company pursuant to this Agreement. In addition, Foundation agrees not to use, and to use Commercially Reasonable Efforts to prevent use by any of its Affiliates, of any material non-public information contained in an M&A Approval Request and/or and M&A Notice for the purposes of transactions involving the equity or debt securities of either (i) Company or its Affiliates or (ii) any entity participating in the potential or actual transactions resulting in the PTC Corporate Change described in such M&A Approval Request and/or and M&A Notice. In addition, following receipt of an M&A Approval Request and/or and M&A Notice that includes material non-public information given while Company is subject to the periodic reporting requirements of Section 13(a) or 15(d) of the Securities Exchange Act of 1934, as amended, and for so long as the material information included in the M&A Approval Request and/or M&A Notice is non-public, unless specifically invited in writing by Company to do so, Foundation shall not, and shall use Commercially Reasonable Efforts to prevent its Affiliates from, in any manner, directly or indirectly, (a) publicly effecting or seeking, initiating, offering or proposing to effect or cause or

participating in (whether publicly or otherwise and whether directly or through a Third Party), any tender or exchange offer, merger, consolidation or other business combination involving Company; or any "solicitation" of "proxies" (as such terms are used in the proxy rules of the United States Securities and Exchange Commission) or consents to vote any voting securities of Company; (b) forming, joining or in any way participating in a "group" (as defined under the Securities Exchange Act of 1934, as amended) with respect to any voting securities of Company (or security convertible into rights to acquire any voting security of Company); (c) taking any action which could reasonably be expected to force Company to make a public announcement regarding any of the types of matters set forth in (a) above; or (d) entering into any agreements, discussions or arrangements with any Third Party with respect to any of the foregoing. In addition, Foundation shall not, and shall use

Commercially Reasonable Efforts to prevent its Affiliates from, causing or knowingly permitting any of its or their respective directors, officers, employees, investment bankers (acting in their capacities on behalf of Foundation or any such Affiliate, as applicable), attorneys (acting in their capacities on behalf of Foundation or any such Affiliate, as applicable), accountants (acting in their capacities on behalf of Foundation or any such Affiliate, as applicable), or other advisors or representatives (acting in their capacities on behalf of Foundation or any such Affiliate, as applicable) to initiate or participate in any of the actions described in the foregoing clauses (a), (b), (c), or (d).

10. Partnering.

(a) Company shall have the primary responsibility to evaluate the need for and timing of Collaboration Activities.

(b) During any period in which Company is not actively pursuing Collaboration Activities, Company shall report to the JSC on [**] basis Company's views of the partnering/collaboration marketplace for drug discovery and lead optimization efforts at a similar stage to efforts under the DC Research or, if a Development Candidate has been declared, the partnering/collaboration marketplace for development candidates at a similar stage of development to such Development Candidate.

(c) If Company determines to actively pursue Collaboration Activities, whether at its own initiative or in response to inquiries from Third Parties, Company will first seek input from the JSC on the nature, scope, and potential terms of a transaction arising in connection with such Collaboration Activities, as well as a rank-ordered summary list of preferred potential counterparties to such transaction. To the extent prepared by Company rather than received by Company from a potential counterparty, Company shall also provide the JSC with an opportunity to review a draft term sheet and related materials in support of its proposed Collaboration Activities. The JSC shall promptly provide input on Company's overall approach to Collaboration Activities, as well as specific input on any term sheet or related materials provided to the JSC.

(d) Prior to Company commencing formal term sheet negotiations or contractual negotiations with any Third Party in connection with Collaboration Activities, Company shall first notify Foundation in writing concerning such negotiations (a "Partnering Notice"). Such Partnering Notice shall be accompanied by any available drafts of term sheets or contracts, or if not available, a summary of the proposed transaction to the extent available, in either case subject to redactions of financial terms to the extent required to comply with any confidentiality agreements with the potential counterparty. Following receipt of a Partnering Notice, Foundation and Company shall have the following rights and obligations in connection with the proposed transaction described in the Partnering Notice (regardless of whether, in the course of negotiations, the terms change from those described the original Partnering Notice):

(i) Foundation shall designate a representative to serve in an advisory capacity with respect such transaction and, if requested by Company, to participate in negotiations subject to appropriate confidentiality protections; *provided, however,* that such representative shall not have the power to commit Foundation to enter into any

amendments to the Agreement absent formal written approval by an appropriately authorized officer of Foundation.

(ii) Foundation may designate legal counsel and [**] of Foundation, each subject to appropriate confidentiality protections, to review and provide comments upon proposed term sheets and contracts for such transaction subject to reasonable time frames consistent with overall progress and status of negotiations and not less than [**]percent ([**]%) of the timeframe specified by Company for the receipt of comments from its senior management. For clarity, such reasonable time frames may be as short as [**] if, in Company's reasonable judgment, such time frames are required to support a successful negotiation process and such time frames are not less than [**] percent ([**]%) of the timeframe specified by Company for the receipt of comments from its senior management. If requested by the potential counterparty, Company shall have the right to redact financial terms from such term sheets and contracts. In reviewing and commenting

on such proposed term sheet and contracts, Foundation counsel and designated representative shall indicate the relative importance of their comments, and if practicable a range of potential responses for negotiation purposes. Company shall use Commercially Reasonable Efforts to implement comments and negotiating positions suggested by Foundation's counsel and/or representative, with due consideration given to the relative importance assigned to the comments and reflecting the outcome of any discussions between Company and Foundation's counsel and/or representative with respect to modification of such comments. In addition, Company shall, as non-negotiable contractual terms, require (1) that the counterparty commit, in response to the JSC's invitation, to sending a representative of such counterparty to such JSC meetings or portions of meetings as the JSC shall request for the purposes of informing, discussing and serving in an advisory role with respect to decisions regarding the progress of and all future plans for the DC Research and the Development and commercialization of the Reversion Candidates, Development Candidate(s) and Product(s) that are the subject of the agreement between Company and such counterparty, (2) an acknowledgement that the JSC shall remain the sole governance body for all research, Development and commercialization decisions regarding the DC Research and the Development and commercialization of Reversion Candidates, Development Candidates and Products that are the subject of the agreement between Company and such counterparty (which acknowledgement may include a clarification that the JSC's role as such sole governance body shall not prevent the Company or its Licensee from making decisions necessary or useful to implement decisions made by the JSC regarding research, Development, commercialization, and Collaboration Activities with respect to Drug Candidates, Reversion Candidates, Development Candidate(s) and Product(s), so long as such implementation decisions are consistent with and faithful to the intent of the JSC's decision), (3) that to the extent the counterparty will assume responsibility for Development of a Development Candidate or Product in any country, that such counterparty assume the obligations and rights of Company pursuant to Second Amendment Section 13 in that country, (4) an acknowledgement that the counterparty's rights and licenses from Company with respect to Reversion Candidates, Development Candidates and Products will terminate upon a Special Termination or Company's receipt of a Reversion Notice or Buy-Out Notice and an obligation in such circumstance for the

counterparty to grant the licenses and rights specified in Section 3 of this Second Amendment and Section 6.1(c)(2) of this Agreement (including licenses and rights to (A) all intellectual property that, if developed, acquired or otherwise Controlled by Company, rather than such counterparty, would be Company Technology or Data ("Licensee Technology" and "Licensee Data", respectively) and (B) all INDs, NDAs or similar regulatory filings made or obtained by such counterparty with respect to the relevant Reversion Candidates, Development Candidates and Products) and perform the activities specified therein in each case as if such counterparty were Company, and (5) third party beneficiary rights for Foundation in the event that such counterparty fails to fulfill any of the foregoing obligations.

(iii) Prior to the conclusion of contractual negotiations pursuant to such Partnering Notice, Company shall schedule at least [**] with representatives of the negotiating team of potential counterparties to the transaction and the representatives designated by Foundation pursuant to the foregoing subsections (i) and (ii) to discuss Foundation's goals and interests with respect to such proposed transaction. Unless otherwise agreed by Foundation, Company shall use Commercially Reasonable Efforts to cause [**] to take place in person at a location convenient to the New York metropolitan area.

(e) Prior to Company entering into a definitive written agreement with any Third Party in connection with Collaboration Activities, Company shall seek the review and approval of the JSC by providing the members of the JSC a proposed final draft of the definitive written agreement and a summary [**] of the proposed transaction, including an overview of any items or terms subject to finalization in the draft provided. If required by the Company's confidentiality agreement with the potential counterparty, Company shall have the right to redact financial terms from such proposed final draft of the definitive written agreement. As promptly as reasonably possible, but in no event later then [**] business days following receipt by the JSC members of such proposed final draft of the definitive written agreement and summary, the JSC shall convene a meeting to either approve or deny for such proposed transaction; *provided, however*, that if Company has otherwise complied with requirements of this Second Amendment Section 10, Foundation shall only be entitled to cast its JSC vote against such proposed transaction if it agrees either (i) to fund [**] percent ([**]%) of ongoing Development and commercialization costs for the applicable Development Candidate(s) or Product(s), or (ii) [**] and any related rights pursuant to Section [**]; and *provided further*, that failure of either party to make itself available within the time frames specified in this Second Amendment Section 10(e) shall entitle the other party to either approve or deny the proposed transaction in the name of the JSC without the requirement of holding an actual JSC meeting. If the JSC denies approval in accordance with this Second Amendment Section 10(e), Company shall not enter into such proposed definitive written agreement, but shall have the right to continue the applicable negotiations consistent with this Second Amendment Section 10 for the purposes of achieving a form of such definitive written agreement acceptable to the JSC.

(f) Following the entry into a transaction pursuant to this Second Amendment Section 10, the following additional terms and conditions will apply:

(i) The JSC shall continue as the sole governance body for the conduct of the DC Research and the Development and commercialization of Reversion Candidates, Development Candidates and Products and shall continue to have all the rights and responsibilities specified in Second Amendment Section 5.

(ii) The JSC shall invite the representative of the counterparty designated pursuant to Second Amendment Section 10(d)(ii)(1) to such JSC meetings, or portions of meetings, as the JSC shall deem advisable for the purposes of informing, discussing and serving in an advisory role with respect to decisions regarding the progress of and all future plans for the DC Research and the Development and commercialization of the Reversion Candidates, Development Candidate(s) and Product(s) that are the subject of the agreement between Company and such counterparty.

11. Company Payments to Foundation and Related Provisions: Sections 4.3, 4.4, 4.5, 4.6 and 4.7 of the Agreement (captioned "Milestone Donation by Company", "Reporting of Product Revenues", "Exchange Rate; Manner and Place of Payment", "Taxes" and "Audits", respectively) shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"4.3 Payments by Company. Company will make the following payments to Foundation in connection with Product Revenues:

"(a) Company will make payments as specified below to Foundation up to a maximum amount equal to [**] by [**] pursuant to the Agreement (the "SMAF Funding Amount," which, for clarity, includes [**] pursuant to the [**] defined below (such total amount, the "Repayment Amount"). For the purposes of this Section 4.3 of the Agreement, the "[**]" shall be [**] unless and until both (i) a Product has achieved Worldwide Net Sales of at least [**] US dollars ($[**]) in any calendar year; and (ii) the SMAF Funding Amount received with respect to such Product equals or exceeds a total of [**] US dollars ($[**]), upon the occurrence of which the [**].

"(b) In the event that Company and/or its Affiliates sells Products, then Company shall pay the Repayment Amount to the Foundation by making installment payments to the Foundation, each of which shall be equal to [**] percent ([**]%) of Net Sales received by Company and its Affiliates in the applicable calendar quarter and each of which shall be paid in U.S. dollars, by wire transfer to an account specified by the Foundation, within [**] days of end of such calendar quarter. The first such installment payment shall be paid for the first calendar quarter following the first calendar year in which Net Sales equaled or exceeded [**] U.S. Dollars (US$[**]) (the "Sales Threshold"). An additional installment payment shall be paid to the Foundation for each subsequent calendar quarter until such time as the sum of all installment payments made pursuant to this Section 4.3(b) of the Agreement, together with all installment payments made pursuant to Section 4.3(c) of the Agreement, equals the Repayment Amount. If the [**], Company shall make additional payments until the updated Repayment Amount has been met.

"(c) In the event that Company and/or its Affiliates enters into one or more license agreements for the development, manufacture, use, distribution, promotion or sale of a Drug Candidate or Product in one or more territories, then Company shall repay the Repayment Amount by making installment payments to the Foundation, each of which shall be equal to [**] percent ([**]%) of PTC License Income received by Company and its Affiliates in the applicable calendar quarter and each of which shall be paid in U.S. dollars, by wire transfer to an account specified by the Foundation, within [**] days of the end of such calendar quarter. The first quarter during which such installment payments shall be paid shall be the first calendar quarter following the first calendar year in which both of the following criteria are met: (i) a Licensee makes or has previously made its First Commercial Sale and (ii) Worldwide Net Sales equal or exceeded [**] U.S. Dollars (US$[**]). An additional installment payment shall be paid to the Foundation for each subsequent calendar quarter until such time as the sum of all installment payments made pursuant to this Section 4.3(c) of the Agreement, together with all installment payments made pursuant to Section 4.3(b) of the Agreement, equals the Repayment Amount. If the [**], Company shall make additional payments until the updated Repayment Amount has been met. Notwithstanding the foregoing, if the payments owed pursuant to this Section 4.3(c) of the Agreement would, when combined with other payments owed by Company to Third Parties in connection with the receipt of such PTC License Income, exceed [**] percent ([**]%) of such PTC License Income, then the payments owed pursuant to this Section 4.3(c) of the Agreement and such other payments owed by the Company to Third Parties shall all be automatically reduced pro rata until the combined payments no longer exceed [**] percent ([**]%) of such PTC License Income; provided, however, that this reduction shall only be available with respect to payments under this Section 4.3(c) of the Agreement if all other payments owed by Company to Third Parties in connection with the receipt of such PTC License Income are also subject to such pro rata reduction.

"4.4 Reporting of Net Sales and PTC License Income. From and after such time as Company first receives any Net Sales or PTC License Income and until such time as Company has paid in full the amount due under Section 4.3 of the Agreement (if any), Company shall deliver to the Foundation (or a Third Party designated in writing by the Foundation) quarterly written reports of Net Sales and PTC License Income received by Company and its Affiliates,

which reports shall (a) separately indicate the total Net Sales and PTC License Income received, (b) show how Net Sales were calculated from the gross amounts received by Company and its Affiliates, with each deduction from gross amounts being separately itemized, (c) show how PTC License Income was calculated, and (d) itemize any amounts received by Company and its Affiliates from a Licensee that were excluded from PTC License Income and the rationale for such exclusion. Company shall keep, and shall cause its Affiliates to keep, complete and accurate records pertaining to the receipt of Net Sales and PTC License Income in sufficient detail to permit the Foundation to confirm the accuracy of such reports.

"4.5 Exchange Rate; Manner and Place of Payment. All payments hereunder shall be payable in U.S. dollars; provided, that in the event that, by reason of applicable legal requirement in any country, it becomes impossible or illegal for a payor to transfer,

or have transferred on their behalf, royalties or other payments to the payee, the payor shall promptly notify the payee of the conditions preventing such transfer and such royalties or other payments shall be deposited in local currency in the relevant country to the credit of the payee in a recognized banking institution designated by the payee or, if none is designated by the payee within a period of [**] days, in a recognized banking institution selected by the payor and identified in a notice given to the payee. When conversion of payments from any foreign currency is required for purposes of a calculation under this Agreement that relates to a payment from one party to the other, such conversion shall be at the exchange rate used by the payor (or, where applicable, a Licensee or licensee of Foundation) throughout its accounting system (which shall, in any event, be commercially reasonable) during the quarter for which such report is due. All payments owed under this Agreement shall be made by check, or by wire transfer in immediately available funds to a bank and account designated in writing by the party entitled to receive payment, unless otherwise specified in writing by such party.

"4.6 Taxes. Each party will pay any and all taxes levied on account of any payments made to it under this Agreement out of the amounts it is to receive hereunder. If any taxes are required to be withheld by the party making payment, such party will (a) deduct such taxes from the payment made by it, (b) timely pay the taxes to the proper taxing authority, (c) send proof of payment to the other party and certify its receipt by the taxing authority within [**] days following such payment, and (d) be deemed to have paid such amount to the other party hereunder.

"4.7 Audits. The Foundation shall have the right to cause an independent, certified public accountant reasonably acceptable to Company to audit the records of Company and its Affiliates to confirm the accuracy of (a) Company's reports of Net Sales and PTC License Income, (b) Company's accounting pursuant to Second Amendment Section 2(d) or 4(a) of its use of internal resources and the out-of-pocket expenses that Company incurred in accordance with the Research Plan, (c) the amount specified in Second Amendment Section 2(d) as the amount spent by Company on DC Research between [**] and [**], and (d) Company's invoice pursuant to Second Amendment Section 2(d) with respect to the amounts it spent between [**] and the Second Amendment Effective Date, in each case for a period covering not more than the preceding [**] years. Such audits may be exercised during normal business hours upon reasonable prior written notice to Company and no more than [**] per year. If an audit reveals that Company has underpaid any amount due to the Foundation, overcharged Foundation pursuant to Second Amendment Section 2(d) or overstated in Second Amendment Section 2(d) the amount that it spent on DC Research between [**] and [**], Company shall pay all such amounts to the Foundation within thirty (30) days of receiving the Foundation's audit report. The Foundation shall bear the full cost of such audit unless such audit discloses (i) an underreporting of Net Sales or PTC License Income by Company of more than [**]% during any calendar year, (ii) an over-reporting of internal resources and the out-of-pocket expenses of more than [**]% during any calendar year or (iii) that Second Amendment Section 2(d) over-states by more than [**]% the amount that Company spent on DC Research between [**] and [**], in which case, Company shall bear the full cost of such audit."

12. Reversionary License. Section 6.1(c) of the Agreement (captioned "Reversionary Licenses to Data and Company Technology") shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"(c) **Reversionary Licenses to Data and Company Technology.**

"(1) In the event that:

"(i) During the term of the DC Research, Company fails to perform its obligations as set forth in Second Amendment Section 2 with respect to conduct of the DC Research, and does not remedy such failure to

comply within [**] days after notice thereof from Foundation; *provided, however,* that in the event [**], the parties shall promptly meet to negotiate in good faith the [**], and Company's right to cure any failure under this Section 6.1(c)(1)(i) shall be extended to the longer of (xx) [**] days after Foundation provides Company written notice that it wishes to terminate such good faith negotiations, or (yy) such other period as the parties may agree in connection with a mutually-agreed plan to address [**];

"(ii) Company is otherwise in material breach of this Agreement with respect to the DC Research and does not remedy such breach within [**] days after notice of such breach from Foundation; or

"(iii) Company is in material breach of its obligations set forth in Section 3.2 of the Agreement, and does not remedy such breach within [**] days after notice thereof from Foundation;

"then, in any such case, Foundation shall have the option to declare the effectiveness of the terms and conditions specified in Section 6.1(c)(2) of the Agreement, such option to be exercised by providing written notice to Company (a "Reversion Notice") within the [**] period following the last date on which Company could have cured such failure or breach pursuant to this Section 6.1(c)(1) of the Agreement.

"(2) Effective upon receipt of a Reversion Notice pursuant to Section 3.2, 3.4(a)(i), 3.4(b), 3.4(c) or 3.4(d) of the Agreement or within the time period specified in Second Amendment Section 3(d) or Section 6.1(c)(1) of the Agreement, or upon the effectiveness of a Buy-Out Notice pursuant to Section 3.3(b)(v) of the Agreement (regardless whether the Foundation obtained its Buy-Out Right pursuant to Section 3.3(b)(iv) of the Agreement, Section 3.4(a)(ii) of the Agreement or Second Amendment Section 9(b)(4)), the following terms and conditions shall apply:

"(i) Company shall, and it hereby does, grant to Foundation an exclusive worldwide license, including the right to grant sublicenses, under any Company Technology, Licensee Technology, Data or Licensee Data that relates to a pharmaceutical preparation, composition of matter, method of manufacture and/or method of use in the Field, of Reversion Candidates and/or Products containing one or more Reversion Candidates, solely for the purpose of

researching, developing, making, having made, using, selling, having sold, offering for sale and importing Reversion Candidates and Products containing Reversion Candidates (such Products, "Reversion Products") in the Field (such license being referred to herein as the "Reversionary License"). The Reversionary License shall be fully-paid up and royalty free unless the Foundation obtains the Reversionary License pursuant to (A) Section 3.3(b)(iv) of the Agreement, Section 3.4(a)(ii) of the Agreement, or Second Amendment Section 9(b)(4), in which case the licensing fees, royalties and other terms set forth in Section 6.1(c)(3) of the Agreement shall also apply or (B) Second Amendment Section 3(d) of the Agreement, in which case the royalties, [**], and other terms set forth in Section 6.1(c)(4) of the Agreement shall also apply;

"(ii) Company shall, and it hereby does, grant to Foundation a fully-paid up, royalty-free, non-exclusive, and worldwide license, including the right to grant sublicenses, to (xx) Company Technology and Licensee Technology to the extent not exclusively licensed pursuant to Section 6.1(c)(2)(i) of the Agreement, (yy) Data and Licensee Data, and (zz) Company Base IP, in each case solely to the extent (1) reasonably necessary for Foundation to exercise its rights under the Reversionary License or (2) useful for Foundation to exercise its rights under the Reversionary License and used or contemplated to be used in the DC Research or pursuant to the Development Plan (as applicable); *provided*, that the license granted to Foundation in this Section 6.1(c)(2)(ii) of the Agreement [**] or [**] or [**];

"(iii) Company shall reasonably cooperate with Foundation in order to enable Foundation to continue, initiate or re-initiate the Development, manufacture and commercialization of the Reversion Candidates or Reversion Products, such cooperation and assistance to be provided in a timely manner (having regard to the nature of the cooperation or assistance requested) and including without limitation (in each case with respect to the Reversion Candidates or Reversion Products): (A) within [**] months of the Reversion Notice or Buy-Out Notice: (1) transferring or granting a right of reference to any INDs, NDAs, or similar regulatory filings made or obtained by Company or its Affiliate or Licensee; (2) providing a copy of all Data and Licensee Data and all reports and other information that were (or should have been) accessible to Foundation via the shared electronic collaboration space described in Second Amendment Section 4(a); and (3) providing reasonable quantities of existing stock of materials (other than (1) materials [**] or (2) materials [**] in Company's possession or under its control and (xx) that are specific to, or were used or were contemplated to be used in, the DC Research or the Development Plan, and are not commercially available from Third Parties, (yy) that are reasonably necessary or useful for continued research, Development or commercialization of Reversion Candidates in the Field, and (zz) the transfer of which would not [**] trigger any contractual obligation to make payments to a Third Party (other

than a Licensee); provided, however, that any subsequent transfers of such materials by Foundation to Third Parties shall be subject to the

terms of a materials transfer agreement reasonably acceptable to Company; (B) permitting Foundation to purchase, for a period of up to [**] years (or less if Foundation obtains an alternative validated, supply source within such [**] year period), Reversion Candidates and Reversion Products [**], but only to the extent (1) such Reversion Candidates or Reversion Products are manufactured by Company itself (as opposed to under a Third Party manufacturing contract) or (2) such Reversion Candidates or Reversion Products are manufactured for the Company by a Third Party and the agreement pursuant to which such Reversion Candidates or Reversion Products are manufactured (xx) provides for manufacture of other active pharmaceutical ingredients or pharmaceutical products that are not Reversion Candidates or Reversion Products, (yy) is not assignable to Foundation or (zz) has not been assigned to Foundation; (C) permitting Foundation to purchase [**] all or any part of Company's worldwide unsold inventory of such Development Candidate or Product together with any raw materials and work-in-process relating to such Development Candidate or Product; (D) upon Foundation's request, using Commercially Reasonable Efforts to assign to Foundation any Third Party manufacturing contracts relating to Reversion Candidate or Reversion Product; (E) upon Foundation's request, using Commercially Reasonable Efforts to assign to Foundation any Third Party license agreements relating to such Reversion Candidate or Reversion Product; and (F) providing prompt technical assistance as requested by Foundation [**] for [**] months after the Reversion Notice or Buy-Out Notice;

"(iv) Foundation, at its own expense, shall maintain clinical trial and/or product liability insurance, as applicable, in an amount consistent with industry standards and only if available on commercially reasonable terms, and shall [**] with respect to such insurance, with respect to losses arising out of or related to its activities pursuant to the Reversionary License and other rights granted in this Section 6.1(c)(2) of the Agreement, and Foundation shall provide a certificate of insurance evidencing such coverage to Company upon request;

"(v) At Foundation's option, on a license-by-license basis, either (i) Foundation may request in writing that Company use Commercially Reasonable Efforts to secure the assignment to Foundation or its designee any licenses granted by Company to Licensees; or (B) upon written notice from Foundation all licenses granted by Company to Licensees shall automatically terminate and Licensees shall be obligated to perform the obligations set forth in this Section 6.1(c)(2) of the Agreement as if they were Company. Company shall include in each agreement with a Licensee an acknowledgement by Licensee of the foregoing and a provision that grants Foundation third party beneficiary status with respect to Licensee's performance (or failure to perform) such obligations;

"(vi) The rights and obligations of the parties pursuant to Article 2 of the Agreement, Article 3 of the Agreement, Sections 4.3, 4.4, 4.5, 4.6, 4.7(a) and 6.3 of the Agreement, First Amendment Sections 5, 6 and 7, and Second Amendment Sections 2 (including Foundation's obligations to fund the DC Research), 3, 4

(except for the final report described in Second Amendment Section 4(b)(ii)), 5, 6, 7, 9, 10, 13, 17, 18(a), 18(b), 18(c), 18(d)and 18(f) shall also terminate; and

"(vii) Notwithstanding the foregoing provisions of Section 6.1(c)(2) of the Agreement, in no event shall Company be required to take any actions pursuant to Section 6.1(c)(2) of the Agreement that, in the good faith judgment of outside counsel to the Company, would infringe any Third Party intellectual property rights (and no non-infringing alternative is identified after a reasonable inquiry) or trigger a breach of any contractual obligations of Company with respect to a Third Party (other than a Licensee)."

"(3) In addition to the provisions of Section 6.1(c)(2) of the Agreement, if Foundation obtains the Reversionary License and other rights set forth in Section 6.1(c)(2) of the Agreement pursuant to Section 3.3(b)(iv) of the Agreement, Section 3.4(a)(ii) of the Agreement, or Second Amendment Section 9(b)(4), then (A) Foundation shall [**] and (B) Foundation shall make the following payments to Company with respect to such Reversionary License and rights:

"(i) If [**] pursuant to this Agreement (which, for clarity, [**] as of the accrual of the applicable Buy-Out Right, then Foundation shall pay to Company a licensing fee equal to [**] U.S. dollars ($[**]) as specified in (iii) below.

"(ii) In the alternative, if [**] with respect to [**] pursuant to this Agreement (which, for clarity, [**]) as of the accrual of the applicable Buy-Out Right, then Foundation shall pay, as specified in (iii) below, to Company a licensing fee that is equal to the sum of [**] U.S. dollars ($[**]) plus *x*, where *x* equals the lesser of (A) [**] and (B) [**] U.S. dollars ($[**]). For clarity, such licensing fee shall never exceed [**] U.S. dollars ($[**]).

"(iii) Foundation shall pay the licensing fee set forth in (i) or (ii) above in three installments: (A) a first installment, equal to [**] percent ([**]%) of such license fee shall be paid by Foundation simultaneously with Foundation's notice that it is exercising such Buy-Out Right; (B) a second installment, equal to [**] percent ([**]%) of such license fee shall be paid by Foundation by the [**]month anniversary of Foundation's notice that it is exercising such Buy-Out Right, provided that Company has complied with its obligations pursuant to Section 6.1(c)(2) of the Agreement in good faith and responded promptly and adequately to any Foundation notices detailing any alleged lack of such good faith compliance; and (C) a final installment, equal to [**] percent ([**]%) of such license fee shall be paid by Foundation (x) by the [**]month anniversary of Foundation's notice that it is exercising such Buy-Out Right, provided that Company has complied with its obligations pursuant to Section 6.1(c)(2) of the Agreement in good faith and responded promptly and adequately to any Foundation notices detailing any alleged lack of such good faith compliance, or (y) if earlier, the date upon which Foundation is satisfied that Company has fully performed all obligations of Company set forth in Section 6.1(c)(2) of the Agreement;

"(iv) [**], Foundation shall pay to Company the following percentage royalties (on a Reversion Product-by-Reversion Product basis) on product revenues (based on the definition of Product Revenues in the Agreement, but substituting "Reversion Product" for "Product" and "Foundation" for "Company" as the context requires) of any Reversion Product, such payments to be made on a quarterly basis in arrears no later than [**] days following the end of the applicable quarter:

Stage of Reversion Candidate Upon Exercise of Buy-Out Right by Foundation	**Royalty on Product Revenues of applicable Reversion Product**
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

"For the purposes of the foregoing table, [**] shall mean [**]. The royalties in the foregoing table shall commence, on a country-by-country basis, upon the first commercial sale (based on the definition of First Commercial Sale in the Agreement, but substituting "Foundation" for "Company" as the context requires) of the applicable Reversion Product in such country, and continue for the longer of (xx) [**] from the date of such first commercial sale or (yy) the date of expiration of the last Company Patent covering the applicable Reversion Product within the applicable country. Foundation shall comply with the applicable provisions of Sections 4.4, 4.5, 4.6 and 4.7 of the Agreement with respect to such royalty payments, substituting "Company" for "Foundation" and vice versa as the context may require."

"(4) In addition to the provisions of Section 6.2(c)(2) of the Agreement, if Foundation obtains the Reversionary License and other rights set forth in Section 6.1(c)(2) of the Agreement pursuant to Section 3.2 of the Agreement or Second Amendment Section 3(d), then Foundation shall make the following payments to Company with respect to the Reversionary License and shall have the following obligations to Company:

"(i) Foundation shall (A) [**] and (B) make royalty payments to Company, on a Reversion Product-by-Reversion Product basis, equal to [**] percent ([**]%) of product revenues (based on the definition of Product Revenues in the Agreement, but substituting Reversion Product for Product and Foundation for Company as the context requires) of any Reversion Product, such payments to

be made on a quarterly basis in arrears no later than [**] days following the end of the applicable quarter. Such royalty payments shall commence, on a country-by-country basis, upon the first commercial sale (based on the definition of First Commercial Sale in the Agreement, but substituting "Foundation" for "Company" as the context requires) of the applicable Reversion Product in such country, and continue for the longer of (xx) [**]

from the date of such first commercial sale or (yy) the date of expiration of the last Company Patent covering the applicable Reversion Product within the applicable country. Foundation shall comply with the applicable provisions of Sections 4.4, 4.5, 4.6 and 4.7 of the Agreement with respect to such royalty payments, substituting "Company" for "Foundation" and vice versa as the context may require."

"(ii) Before granting any Third Party an exclusive sublicense of the Reversionary License for any purpose that includes commercializing any Reversion Product in the United States, [**], for a period of up to [**] months, [**] would be [**] and [**] to [**]. If the [**], by the end of such [**] month period, a [**] that set[**], then Foundation shall be free to grant such a sublicense to a Third Party [**]."

"(5) If Foundation makes a final decision, with respect to each and every Reversion Candidate, that it has no interest in performing or having performed (including through a sublicensee), at such time or at any point in the future, any further research, Development, or commercialization upon such Reversion Candidate pursuant to the Reversionary License, then it shall provide written notice thereof to Company, and Company shall be entitled to [**] and this Agreement on written notice to Foundation."

13. Clinical Trials and Access to Materials. The terms and conditions of this Second Amendment Section 13 shall apply equally to each Licensee as if such Licensee were Company, shall be included in the agreement pursuant to which Company grants rights to such Licensee with respect to any Drug Candidate or Product, and Foundation shall be a third party beneficiary with respect to such terms and conditions and shall have the right to take action directly against such Licensee if such Licensee fails to comply with such terms and conditions.

(a) SMAF Clinical Trials Advisory Committee. Foundation shall have the right, but not the obligation, to create a committee of experts to advise Foundation and Company on clinical trials and expanded access with respect to Development Candidates and Products (the "SMAF Clinical Trials Advisory Committee"). Such SMAF Clinical Trials Advisory Committee shall consist of such individuals as Foundation may designate, but shall include at least one clinical investigator with experience in the Field, [**]. The SMAF Clinical Trials Advisory Committee shall have, as one of its principal mandates, the responsibility of balancing (i) the rapid and efficient Development and commercialization of Development Candidates and Products for the benefit of all potential patients in the Field and (ii) the appropriateness, based on available safety and efficacy information with respect to such Development Candidates and Products, of providing access to such Development Candidates or Products to individual patients via the extension protocols to Company Clinical Trials or expanded access programs further

described in Second Amendment Sections 13(b) and 13(c). Such SMAF Clinical Trials Advisory Committee may establish its own procedures for meetings and decision-making.

(b) Company Clinical Trials.

(1) Foundation shall have the right, but not the obligation, to assist with patient recruitment for any Company Clinical Trial involving SMA patients by (i) referring to Company (or, at Company's request, referring directly to any clinical investigator at a clinical trial site for the applicable Company Clinical Trial) up to [**] SMA patients meeting the enrollment criteria for the applicable Company Clinical Trial and identified by Foundation or its designee, and/or (ii) proposing up to [**] clinical trial sites with access to appropriate patient populations for such Company Clinical Trial. Company shall use Commercially Reasonable Efforts to enable such patients to be enrolled in such Company Clinical Trial consistent with the applicable enrollment criteria, protocol, and target patient number for such Company Clinical Trial (it being understood that such patients should be given priority over other patients who are equally qualified to participate in such Company Clinical Trial, provided that the final decision regarding such enrollment is made by the clinical investigator and/or clinical trial site personnel of the investigating institution), and to contract with such clinical trial sites for such Company Clinical Trial. If Foundation, in its sole discretion, determines not to assist in patient recruitment for any Company Clinical Trial, then it shall so inform Company and Company shall assume all responsibility for patient recruitment and selection of clinical trial sites.

(2) Each time that Company commences the drafting of a clinical trial protocol for a Development Candidate or Product and at reasonable times thereafter, Company will discuss with Foundation Company's plans for making such Development Candidate or Product available to participants in such clinical trial after the completion of such trial. If mutually agreed by the parties based on such discussions, or if recommended by the SMAF Clinical Trials Advisory Committee in its sole discretion, Company will submit to the appropriate Regulatory Agency a suitable extension protocol and corresponding informed consent form providing for administration of such Drug Candidate or Product for at least [**] beyond the term provided for in a particular Company Clinical Trial. Company shall use Commercially Reasonable Efforts to obtain the applicable Regulatory Agency's approval of such extension protocol and informed consent and subsequent approval from the Institutional Review Boards at the locations where such Company Clinical Trial is being conducted; *provided, however*, that the proposed [**] period for such extension protocol may be shortened based on the request or advice of the applicable Regulatory Agency. Upon receipt of such approvals, Company shall provide, in accordance with the approved extension protocol, such Development Candidate or Product to

those SMA patients who enrolled in such Company Clinical Trial pursuant to this Second Amendment Section 13(b) and wish to continue to receive such Development Candidate or Product after the completion of such Company Clinical Trial (such patients, the "Enrollees"). For so long as Company is continuing to develop or seek approval from a Regulatory Agency for such Development Candidate or Product, and subject either to mutual agreement of Company and Foundation or to the recommendation of the SMAF Clinical Trials Advisory Committee in its sole discretion, Company shall use commercially reasonably efforts to obtain approval for an amended or new extension protocol providing for continued administration of such Development Candidate or Product to the Enrollees, and Company shall

provide such Development Candidate or Product to the Enrollees in accordance with any such approved protocol. In any case in which Company, [**], does not concur in the decision to commence or continue any extension protocols pursuant to this Second Amendment Section 13(b)(2), then Company's obligations to assist with such extension protocols and continue to supply such Development Candidate or Product to Enrollees shall [**], directly or indirectly, [**], and [**] of Development Candidate or Product to Enrollees.

(3) If Company stops Developing or seeking approval from a Regulatory Agency of a Development Candidate or Product for which it filed an extension protocol pursuant to Second Amendment Section 13(b)(2), and either the parties mutually agree or the SMAF Clinical Trials Advisory Committee in its sole discretion (but having considered any safety issues) recommends that the Enrollees continue to have access to such Development Candidate or Product for a longer period than provided for in any existing extension protocol submitted by Company with respect to such Development Candidate or Product, then upon Foundation's request, Company shall facilitate Foundation's efforts to arrange for prolonged continued access to such Development Candidate or Product for some or all of the Enrollees by taking all reasonable actions requested by Foundation (consistent with the SMAF Clinical Trials Advisory Committee's recommendations, if applicable), including without limitation: (i) either (1) transferring Company's IND for such Development Candidate or Product to Foundation or its designee or (2) providing Foundation or its designee with a right of reference to the manufacturing-related information and safety and efficacy data in Company's IND or Drug Master File or equivalent regulatory filing (as applicable) so that Foundation or its designee can submit its own IND with respect to such continued access; (ii) providing (for the shorter of [**] months or the amount of time necessary for Foundation or its designee to establish an alternative supply of equivalent clinical grade product) such Development Candidate or Product to Foundation or its designee for administration to such Enrollees in accordance with any extension protocol for which Foundation or its designee has obtained approval from the FDA or the applicable Agency; (iii) assisting Foundation or its designee with obtaining an alternative, equivalent clinical grade supply of such Development Candidate or Product by (1) facilitating Foundation's or its designee's negotiation of a supply agreement with Company's manufacturer of such Development Candidate or Product or (2) providing technology transfer and other technical assistance reasonably requested by Foundation to enable Foundation or its designee to manufacture such Development Candidate or Product; and (iv) providing Foundation with a non-exclusive, fully paid, sublicensable license under Company Technology and Data, and solely to the extent reasonably necessary for Foundation to exercise its rights under the foregoing license, to Company Base IP (*provided*, that the license granted hereunder to Foundation [**] or [**] or [**] to perform or have performed on its behalf any and all activities necessary or reasonably useful to provide continued access to such Development Candidate or Product in accordance with this Second Amendment Section 13(b)(3). In any case in which Company, [**], does not concur in the decision to commence or continue any extension protocols pursuant to this Second Amendment Section 13(b)(3), then Company's obligations to assist with such extension protocols and continue to supply such Development Candidate or Product to Enrollees shall [**], directly or indirectly, [**], and [**] of Development Candidate or Product to Enrollees. In connection with the foregoing, Foundation, [**], shall maintain clinical trial and/or product liability insurance, as applicable, in an amount consistent with industry standards and only if available on commercially reasonable terms, and shall [**] with respect to such insurance, with

respect to losses arising out of or related to the activities contemplated under this Second Amendment Section 13(b)(3). Foundation shall provide a certificate of insurance evidencing such coverage to Company upon request.

(c) Expanded Access Program.

(1) At such a time as the parties mutually agree, or the SMAF Clinical Trials Advisory Committee in its sole discretion determines, that results from Company Clinical Trials and other Development activities with respect to the applicable Development Candidate or Product support expanded access to such Development Candidate or Product for patients with SMA, then Company and Foundation shall cooperate to establish such an expanded access program in which at least [**] SMA patients identified by Foundation who do not meet the enrollment criteria for a particular Company Clinical Trial (whether or not such Company Clinical Trial is directed to SMA patients) for such Development Candidate or Product (such patients, the "Patients") may gain access to such Development Candidate or Product. Company agrees that at its earliest reasonable opportunity following the commencement of such cooperation (e.g., at a meeting with FDA), Company will inquire about the feasibility of an

expanded access protocol for such Drug Candidate or Product for SMA purposes and will invite a designee of Foundation with appropriate medical or regulatory experience to participate in discussions with the FDA regarding the establishment and maintenance of such expanded access program. In connection with such expanded access program, at Foundation's request and consistent with any recommendation made by the SMAF Clinical Trials Advisory Committee, Company will either (i) submit to the FDA a protocol that is reasonably acceptable to Foundation and calls for administering such Development Candidate or Product to the Patients or (ii) notify Foundation that it will not be making such a submission and facilitate the submission and approval of such a protocol by the Foundation or its designee.

(2) If Company chooses option (i) above, then it shall use Commercially Reasonable Efforts to obtain approval of such protocol and upon receipt of such approval, it shall provide such Development Candidate or Product to the Patients in accordance with the approved protocol; *provided*, that the parties shall engage in good faith negotiations with respect to [**].

(3) If Company chooses option (ii) above, then Company shall facilitate Foundation's efforts to arrange for such expanded access program for such Development Candidate or Product for the Patients by taking all reasonable actions requested by Foundation, in each case [**], including without limitation: (1) either (1) allowing the expanded access program to be performed pursuant to Company's IND (in which case Foundation or its designee shall provide Company with all data arising from and other information with respect to such expanded access program that is necessary or reasonably useful for Company to fulfill its obligations as the IND holder) or (2) providing Foundation or its designee with a right of reference to the manufacturing-related information and safety and efficacy data in Company's IND or Drug Master File or similar regulatory filing (as applicable) so that Foundation or its designee can file its own IND with respect to such expanded access program; (ii) providing such Development Candidate or Product to an appropriate designee of Foundation for administration to the Patients in accordance with any expanded access protocol for which Foundation or its designee has obtained approval from the FDA [**]); and (iii) providing Foundation with a non-

exclusive, fully paid, sublicensable license under Company Technology and Data, and solely to the extent reasonably necessary for Foundation to exercise its rights under the foregoing license, to Company Base IP (*provided*, that the license granted to Foundation hereunder [**] or [**] or [**] to perform or have performed on its behalf any and all activities necessary or reasonably useful to provide such expanded access to such Drug Candidate or Product in accordance with this Second Amendment Section 13(c)(3). In connection with the foregoing, Foundation, [**], shall maintain clinical trial and/or product liability insurance, as applicable, in an amount consistent with industry standards and only if available on commercially reasonable terms, and shall [**] with respect to such insurance, with respect to losses arising out of or related to the activities contemplated under this Second Amendment Section 13(c)(3). Foundation shall provide a certificate of insurance evidencing such coverage to Company upon request.

(d) Indemnification by Foundation. In connection with the foregoing Second Amendment Sections 13(b)(3) and 13(c)(3), Foundation hereby agrees to save, defend, indemnify and hold harmless Company, its trustees, officers, employees and agents (each, a "Company Indemnitee") from and against any and all losses, damages, liabilities, expenses and costs, including reasonable legal expenses and attorneys' fees ("Company Losses"), to which a Company Indemnitee may become subject as a result of any claim, demand, action or other proceeding by any Third Party to the extent such Company Losses arise directly or indirectly out of (a) [**] or [**] of any Development Candidate or Product by Foundation, its Affiliate(s) or Licensee(s) pursuant to Second Amendment Sections 13(b)(3) or 13(c)(3), or (b) the breach of this Agreement by Foundation or the gross negligence or willful misconduct of Foundation pursuant to Second Amendment Sections 13(b)(3) or 13(c)(3), except in each case to the extent such Losses result from (x) the breach of this Agreement by Company or the gross negligence or willful misconduct of any Company Indemnitee, or (y) the activities of Company or its agents or employees in connection with any Development Candidate or Product. The obligations of Foundation under this Second Amendment Section 13(d) are conditioned upon Company's delivery of written notice to Foundation of any potential Company Losses promptly after Company becomes aware of such potential Company Losses. Foundation shall have the right to assume the defense of any suit or claim related to Company Losses if it has assumed responsibility for the suit or claim in writing. If Foundation defends the suit or claim, Company may participate in (but not control) the defense thereof at its sole cost and expense but Company may not settle such suit or claim without the prior written consent of Foundation, not to be unreasonably withheld.

(e) Clinical Trial/CRO Agreements. In connection with the foregoing Second Amendment Sections 13(b)(3) and 13(c)(3), Foundation hereby agrees that under any circumstance in which Foundation is contracting directly with clinical trial sites, clinical investigators, and contract research organizations ("CROs"), it will use as the basis for its negotiations [**], and will use Commercially Reasonable Efforts to secure terms with respect to publication, confidentiality, intellectual property (which shall be [**], as the case may be, [**]), and indemnification substantially similar to those routinely obtained by Company with respect to such an agreement, and naming the Company as a third-party beneficiary.

14. Patents. Section 6.2 of the Agreement (captioned "Patent Filings") shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"6.2. Patent Filings.

"(a) Company shall control the filing, prosecution and maintenance of all Patents on Company Technology at its sole expense, which expense shall be included as part of Company's contribution to the Research Project and not payable or reimbursable by Foundation; *provided,* that Foundation shall have reasonable rights of comment and consultation on all such filing, prosecution and maintenance activities; and *provided further* that with respect to initial filings claiming the composition of matter, method of use, or process for manufacturing small molecules, Foundation's review shall be confined to specific individuals reasonably acceptable to Company.

"(b) Subject to the prior written consent of Foundation, such consent not to be unreasonably withheld, delayed or conditioned, Company shall have the right to disclose, in connection with the filing, prosecution or maintenance of any Patents on Company Technology filed by it pursuant to this Agreement, any Confidential Information to the extent reasonably necessary to support and enable the claims of any application with respect to such Patents, or to maintain or enforce any such issued Patents. If, with respect to a specific filing or other document to be submitted to a governmental or quasi-governmental authority in connection with an issued Patent or application for a Patent, Foundation has reviewed and commented on such filing or document pursuant to Section 6.2(a) of the Agreement and raised no objections to the use of Confidential Information, then such consent will be deemed to have been granted for such filing or document.

"(c) Notwithstanding the foregoing Sections 6.2(a) and 6.2(b) of the Agreement, if Company grants the Reversionary License to Foundation pursuant to Section 6.1(c)(2) of the Agreement with respect to a Reversion Candidate or Reversion Product, then Foundation (or its designee) shall have the rights and obligations of Company under Sections 6.2(a) and 6.2(b) of the Agreement (substituting "Foundation" for "Company" as the context requires) with respect to Patents exclusively licensed to Foundation pursuant to such Reversionary License; *provided, however*, that [**], and [**] of the Agreement.

"(d) Each of Company and Foundation shall execute all papers and instruments, and require its employees and contractors to execute all papers and instruments, so as to enable the other party to exercise the rights set forth in this Section 6.2 of the Agreement."

15. Confidentiality and Exceptions. Section 5.1 of the Agreement (captioned "Confidentiality") and Section 5.2 of the Agreement (captioned "Exceptions") shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"5.1 Confidentiality. Except to the extent expressly authorized by this Agreement or otherwise agreed in writing by the parties, the parties agree that, during the Term and for a period of [**] years thereafter, each party (the ***"Receiving Party"***) will maintain in confidence all Confidential Information disclosed to it by the other party (the ***"Disclosing Party"***), provided that, with regard to Confidential Information which is trade secret information, such obligation shall extend thereafter until such information is

no longer a trade secret of the Disclosing Party. The Receiving Party may use the Confidential Information of the Disclosing Party only to the extent required to accomplish the purposes of this Agreement. The Receiving Party shall use at least the same standard of care as it uses to protect proprietary or confidential information of its own to ensure that its employees, agents, consultants and other representatives do not disclose or make any unauthorized use of the Disclosing Party's Confidential Information; provided, however, each party shall ensure that any such employees, agents, consultants and other representatives who are granted access to trade secrets or, prior to publication, to other potentially patentable matter for which patent protection has been or is planned to be sought, arising from the DC Research shall sign written agreements containing confidentiality obligations substantially similar to those set forth in this Agreement except that the duration of such confidentiality obligations for consultants may be less than the duration set forth in this Agreement provided that the duration shall be for a minimum of [**] years from the date of disclosure. Each party will promptly notify the other upon discovery of any unauthorized use or disclosure of the other party's Confidential Information.

"5.2 Exceptions. The obligations of non-disclosure and non-use contained in Section 5.1 will not apply to the extent that it can be established by the Receiving Party by competent proof that such Confidential Information: (a) was already known to the Receiving Party, other than under an obligation of confidentiality, at the time of disclosure by the Disclosing Party; (b) was generally available to the public or otherwise part of the public domain at the time of its disclosure to the Receiving Party; (c) became generally available to the public or otherwise part of the public domain after its disclosure and other than through any act or omission of the Receiving Party in breach of this Agreement; (d) is

independently discovered or developed by the Receiving Party without the use of Confidential Information of the Disclosing Party; or (e) was disclosed to the Receiving Party, other than under an obligation of confidentiality, by a Third Party who had no obligation to the Disclosing Party not to disclose such information to others."

16. Publications, Presentations and Public Disclosures. Section 5.4 of the Agreement (captioned "Publication") and Section 5.5 of the Agreement (captioned "Publicity; Regulatory Disclosures") shall, as of the Second Amendment Effective Date, be amended and restated as follows:

"5.4. Scientific and Medical Publications and Presentations.

"(a) Company and Foundation each acknowledge the other party's interest in publishing or presenting certain results of the Research (including but not limited to the DC Research) to obtain recognition within the scientific community and to advance the state of scientific knowledge and enhance the progress of research in the Field, in all cases in a manner consistent with existing obligations to Third Parties and scientific and industry standards for the research, development and commercialization of small molecules for the treatment, mitigation or prevention of disease. Each party also recognizes their mutual interest in obtaining Patents in support of Products, and the need for such publications or presentations to be strictly monitored to prevent any adverse

effect from premature publication or dissemination of results of the activities hereunder. Consequently, the JSC shall establish reasonable policies and procedures with respect to scientific publications and presentations that balance the foregoing interests, and once established both parties shall be bound by such policies and procedures, and may establish a separate publication committee to administer such policies and procedures. In the event no such procedures and policies are established by the JSC, either party, its employees or consultants wishing to make a publication in a scientific or medical journal or a presentation or similar oral disclosure made at a scientific or medical conference without obligation of confidentiality relating to work performed as part of the Research (the "Publishing Party") shall transmit to the other party (the "Reviewing Party") a copy of the proposed written publication or a written detailed description of the proposed oral disclosure at least [**] days prior to submission or disclosure (or, in the case of Third Party agreements, such shorter period as required by such Third Party agreement) prior to submission for publication or presentation. The Reviewing Party shall have the right (a) to make modifications to the publication for accuracy or intellectual property reasons, and (b) to obtain a delay in publication or presentation of up to [**] days (or, in the case of Third Party agreements, such shorter period as required by such Third Party agreement) in order to enable patent applications or similar applications protecting rights in such information to be filed, and each party shall have the right to prohibit disclosure of any of its Confidential Information (except as otherwise provided in Section 6.2 of the Agreement) in any such proposed publication or presentation. Notwithstanding the foregoing, in no event shall any publication, presentation, or other public disclosure disclose the chemical structure of any Lead Candidate, Drug Candidate, Development Candidate, or Product absent specific permission from the JSC. In any permitted publication or presentation by a party, the other party's contribution shall be duly recognized, and authorship shall be determined in accordance with customary practice in the scientific or medical field.

(b) Company shall provide in each Research Report a summary section which is suitable for immediate public disclosure and the Foundation may release copies of such portions of each Research Report and supporting Data other than chemical structures to any Third Party investigator who requests such material from the Foundation in writing; *provided, however,* that said Third Party investigator first executes Company's non-disclosure agreement that the Company provides to the Foundation for such purpose (it being understood that such non-disclosure agreement will not prohibit said Third Party investigator from applying his or her knowledge of the Data to further SMA research and/or to treatment of SMA patients, but will prohibit him or her from transferring such Data except as incidental and necessary to treating SMA patients).

(c) The parties acknowledge that during the course of research, development and commercialization of Products, it may be necessary to enter into agreements with Third Parties that require different standards for publication and presentation of research results relating to the Research. Notwithstanding Section 5.4(a) of the Agreement, the party conducting research, development or commercialization of Products may enter into agreements with academic, government, nonprofit or similar entities which allow principal investigators and other external researchers to publish or present the results of

their research on terms inconsistent with Section 5.4(a) of the Agreement; provided, that each party entering into such agreements shall use Commercially Reasonable Efforts to include provisions reasonably consistent with and similar to

those appearing in Section 5.4(a) of the Agreement in such Third Party agreements.

"5.5 Publicity; Regulatory Disclosures. In connection with the execution of the Second Amendment, the parties shall jointly issue one or more press releases, the contents of which shall be mutually agreed. Except as otherwise required by law or regulation, or as permitted pursuant to Section 5.4 of the Agreement, neither party shall issue any additional press release or make any other public disclosure concerning this Agreement or the subject matter hereof without first providing the other party with a copy of the proposed release or public disclosure for review and comment, provided that such right of review and comment shall only apply for the first time that specific information is to be disclosed, and shall not apply to the subsequent disclosure of substantially similar information that has previously been disclosed. The party proposing to make the press release or other public disclosure shall give due consideration to any reasonable comments by the other party relating to such proposed press release or other public disclosure. The principles to be observed by the parties in press releases or other public disclosures with respect to this Agreement shall be: accuracy, compliance with applicable legal and regulatory requirements, the requirements of confidentiality under this Agreement and customary business practice in the biopharmaceutical industry for disclosures by companies comparable to Company. For the avoidance of doubt, either party may issue such press releases as it determines, based on advice of counsel, are reasonably necessary to comply with laws or regulations (including regulations of non-governmental regulatory bodies) or for appropriate market disclosure. It is understood, however, that unless required by law or regulation, the parties shall not disclose the specific financial terms and conditions of this Agreement in any press release or other public disclosure. In addition, if a public disclosure is required by law or regulation, including without limitation in a filing with the United States Securities and Exchange Commission, the disclosing party shall provide copies of the proposed disclosure reasonably in advance of such filing or other disclosure for the non-disclosing party's prior review and comment and shall give due consideration to any reasonable comments by the non-filing party relating to such filing, including without limitation the provisions of this Agreement for which confidential treatment should be sought."

17. Baseball Arbitration.

(a) An arbitration under this Second Amendment Section 17 (a "Baseball Arbitration") shall be initiated by written notice of one party to the other and may only be initiated with respect to disputes which meet both the following criteria: (i) the dispute arises from matters within the jurisdiction of the JSC following escalation to the respective Chief Executive Officers of the parties as provided elsewhere in the Agreement, and (ii) Baseball Arbitration is explicitly specified as the method for resolving such dispute pursuant to the Agreement.

(b) The Baseball Arbitration shall be held in a location mutually agreeable to the parties, or if no such location can be agreed, in New York City, according to the then-current commercial arbitration rules of the American Arbitration Association ("AAA"), except to the extent such rules are inconsistent with this Second Amendment Section 17.

(c) The Baseball Arbitration will be conducted by one (1) arbitrator who shall be reasonably acceptable to the parties and who shall be appointed in accordance with AAA rules. If the parties are unable to select an arbitrator within [**] days of the notice that initiated the Baseball Arbitration, then the arbitrator shall be appointed in accordance with AAA rules. Any arbitrator chosen hereunder shall have educational training and industry experience sufficient to demonstrate a reasonable level of scientific, financial, medical and industry knowledge relevant to the particular dispute.

(d) Within [**] days after the selection of the arbitrator, each party shall submit to the arbitrator and the other party a proposed resolution of the dispute that is the subject of the arbitration, together with any relevant evidence in support thereof (the "Proposals"). Within [**] business days after the delivery of the last Proposal to the arbitrator, each party may submit a written rebuttal of the other party's Proposal and may also amend and re-submit its original Proposal. The parties and the arbitrator shall meet within [**] business days after the parties have submitted their final Proposals (and rebuttals, if any), at which time each party shall have [**] to argue in support of its Proposal. The parties shall [**]. Within [**] days after such meeting, the arbitrator shall select one of the final Proposals so submitted by one of the parties as the resolution of the dispute, but may not alter the terms of either final Proposal and may not resolve the dispute in a manner other than by selection of one of the submitted final Proposals. If a party fails to submit a Proposal within the initial [**] day time frame set forth in the first sentence of this Second Amendment Section 17, the arbitrator shall select the Proposal of the other party as the resolution of the dispute. Any time period set forth in this Second Amendment Section 17 may be extended by mutual agreement of the parties.

(e) No arbitrator shall have the power to award punitive damages under this Agreement regardless of whether any such damages are contained in a Proposal, and such award is expressly prohibited. The proceedings and decisions of the arbitrator shall be confidential, final and binding on the parties. Judgment on the award so rendered may be entered in a court having jurisdiction thereof.

(f) [**] the costs of such Baseball Arbitration.

18. Miscellaneous.

(a) Compounds not Selected: During the Second Amendment Term and for the [**] period thereafter, should Company require additional funds for the conduct of research or Development in the Field of any Lead Candidate that was tested in the course of the DC Research but was not selected as a Reversion Candidate (such research and Development, "Non-DC Research"), Foundation will be consulted and provided the opportunity to fund such Non-DC Research in whole or in part prior to any fundraising efforts for such Non-DC Research. Should Company identify an opportunity for agreement with any Third Party or Third Parties with respect to such Non-DC Research during such [**] period, it will provide reasonable advance

notice to Foundation, and the parties will negotiate in good faith (involving such Third Party or Third Parties as appropriate) to develop a structure that supports such additional funding, based on the following principles: (a) entities co-funding such Non-DC Research should share information on the Non-DC Research with each other, subject to appropriate confidentiality provisions, (b) governance with respect to co-funded Non-DC Research should be via a joint steering committee including representatives of Foundation, Company, and any Third Parties, and (c) entities that have provided funding to such co-funded Non-DC Research should have an opportunity (subject to compliance with the terms of their respective funding agreements) to continue their support of such Non-DC Research. For clarity, Company's obligations under this Second Amendment Section 18(a) shall in no way limit Company's ability to engage in general fund-raising activities and to enter into agreements relating thereto.

(b) Additional Testing of Reversion Candidates. After the end of the Research Term and selection of one or more Development Candidates, the JSC (at the request of either party) shall consider whether further research or pre-clinical Development on any Reversion Candidate that is not a Development Candidate is advisable for the purposes of enhancing the utility of such Reversion Candidate as a potential back-up compound or next-generation Product. If deemed advisable by the JSC, and subject to agreement between the parties with respect to funding, Company shall make such Reversion Candidates available for such further research or pre-clinical Development under the terms of a commercially reasonable materials transfer agreement (or a more comprehensive agreement agreed by the parties with respect to such Third Party, which shall include commercially reasonable terms with respect to materials transfer).Without limiting the generality of the foregoing, each such materials transfer agreement shall provide that in no event shall any compound become the property of such Third Party, nor shall any such compound become subject to royalty or other reach-through payment obligations to such Third Party as a result of such testing by such Third Party, and shall also require that a summary of the results of the research be provided to the Company. Company shall share all such research results with the JSC.

(c) Testing by Foundation Partners. Upon the Foundation's request, and under the supervision of the JSC with respect to design of the testing to be done and selection of appropriate compounds and (only during the Research Term) consistent with the then-current Research Plan, Company shall provide reasonable quantities of compounds synthesized or tested during the DC Research ("Research Compounds") to other Foundation partners for testing on a blinded basis in assays already being run by such Foundation partner, provided that (i) after the end of the Research Term, such testing shall be limited to Reversion Candidates and shall not include, without the prior written consent of Company, any Development Candidate that is, at such time, the subject of a Company Clinical Trial, (ii) the Foundation or such Foundation partner shall disclose the results of such screening to Company and (iii) such testing shall be performed pursuant to a separate materials transfer agreement reasonably acceptable to Company and negotiated in good faith by the parties prior to provision of any Research Compounds or related information, which agreement shall contain reasonable and customary terms to protect the parties' respective intellectual property rights. Without limiting the generality of the foregoing, each such materials transfer agreement shall provide that in no event shall any Research Compound become the property of the Foundation partner, nor shall any Research Compound become subject to royalty or other reach-through payment obligations to the

Foundation partner as a result of such testing by such Foundation partner, and shall also require that a summary of the results of the research be provided to the Foundation. Foundation shall share all such research results with the JSC on a regular basis.

(d) Financial Reporting. For so long as it is not a publicly-traded company, (i) Company shall provide Foundation, within [**] days after the end of each of the first three quarters of the fiscal year of the Company, with a copy of the financial report for such quarter that Company generates for its investors, and (ii) Company shall use best its efforts to provide within [**] days, but in no event more than [**] days, after the end of the fiscal year of the Company, a copy of the annual audit report for such year that the Company generates for its investors. The financial reports provided pursuant to Second Amendment Section 18(d)(i) shall be prepared in accordance with generally accepted accounting principles consistently applied, and duly certified (subject to year-end audit adjustments) by the chief financial officer of the Company, and the annual audit report provided pursuant to Second Amendment Section 18(d)(ii) shall be duly certified by independent public accountants of recognized standing. [**], Company may request Foundation consider amending the provisions of this Second Amendment Section 18(d), and Foundation shall consider such request in good faith.

(e) Representations, Warranties and Covenants. Each party hereby represents, warrants and covenants to the other that (i) it has the authority and right to enter into this Second Amendment and to perform its obligations hereunder and (ii) it has not granted as of the Second Amendment Effective Date, and will not grant during the Term (except as specifically allowed and consistent with the applicable terms of this Agreement), any assignment, license, covenant not to sue, option to obtain a license or other right, interest or benefit, exclusive or otherwise, to any Third Party relating to the DC Research or any Reversion Candidate, Drug Candidate, Development Candidate or Product that conflicts with or limits the rights granted to or exercisable by the other party hereunder.

(f) Designation of Third Party Representatives. In any case under this Agreement under which Foundation is entitled to designate a representative, such representative may be a person other than a Foundation employee provided the following criteria are met: (i) Foundation identifies such person in writing to Company, [**], and requests Company's written consent for such addition, such consent not to be unreasonably withheld or delayed and to be based solely on objective written criteria determined by the JSC within [**] months after the Second Amendment Effective Date, which criteria shall vary depending upon the information to which such person is anticipated to have access to in the course of his or her service as such representative; and (ii) such person signs a confidentiality agreement with Company substantially in the form attached as Exhibit SA-7. The JSC shall also establish policies and procedures regarding continuing disclosure obligations for such representatives with respect to conflicts of interest within [**] months of the Second Amendment Effective Date. A breach by a designated representative of Foundation of such conflict of interest policies, or of the confidentiality agreement between Company and such representative, shall entitle Company to terminate such representative effective upon written notice by Company to Foundation stating the grounds for such termination.

(g) Corrective Amendments.

(i) The following Section of the Agreement is cancelled and of no further force and effect: Section 1.20 of the Agreement, the last sentence of Section 2.4(b) of the Agreement, the Option defined in First Amendment Section 2, and First Amendment Section 8.

(ii) Section 2.8 of the Agreement (captioned "Subcontracts") is amended and restated as follows:

"2.8 Subcontracts. Company may perform some of its obligations under the Research Plan through one or more subcontractors, provided that (a) the Research Plan calls for such activities to be subcontracted, (b) none of the rights of either party hereunder are diminished or otherwise adversely affected as a result of such subcontracting, and (c) Company will at all times be responsible for the performance and, except as otherwise agreed by the parties in writing, payment of such subcontractor. In determining whether any Company obligations under the Research Plan will be performed in-house or by a Third Party subcontractor, Company shall take into consideration Company's then-current capabilities, the relative efficiency of utilizing such internal capabilities versus Third Party services and guidance from the JSC."

(iii) Section 6.1(a) of the Agreement (captioned "Data") is amended and restated as follows:

"(a) Data. Company shall solely own all Data."

(iv) Section 6.4 of the Agreement (captioned "No Other License") is amended and restated as follows:

"6.4 No Other License. Other than any licenses granted pursuant to Section 6.1(c) and Second Amendment Section 3 or 13, no license is granted or implied with respect to any Company Technology, Company Base IP or Data for any use."

(v) Section 7.1 of the Agreement (captioned "Term") is amended and restated as follows:

"7.1 Term. The term of this Agreement (the "Term") shall commence on the Effective Date and shall continue until the earliest of: (a) Foundation's receipt of the Repayment Amount in full (including any subsequent payments due on account of [**]); (b) if Foundation exercises a Buy-Out Right, Company's receipt of all payments due pursuant to Section 6.1(c)(3) of the Agreement; (c) if Foundation obtains a Reversionary License pursuant to Section 3.2 of the Agreement or Second Amendment Section 3(d), Company's receipt of all payments due pursuant to Section 6.1(c)(4) of the Agreement; (d) if Foundation obtains a Reversionary License other than as a result of the exercise of a Buy-Out Right or pursuant to Section 3.2 of the Agreement or Second Amendment Section 3(d), the expiration of the last-to-expire Patent licensed to Foundation pursuant to such Reversionary License; or (e) the effective date of any termination in

accordance with this Article 7. For clarity, a Special Termination shall not terminate the term of this Agreement."

(vi) The last sentence of Section 7.3 of the Agreement (captioned "Termination Upon Principal Scientist's Unavailability") is amended and restated as follows: "In the event of a termination of this Agreement pursuant to this Section 7.3 of the Agreement, and notwithstanding any other provision of this Agreement to the contrary (including but not limited to Section 7.4 of the Agreement), only the provisions of Sections 4.7, 6.1(a), 6.1(b), 6.1(c)(2), 6.2(a), 6.2(b), 6.2(d), 7.3, 7.5 of the Agreement, Articles 1, 5, 8, and 9 of the Agreement and Sections 1, 4(b)(ii), and 18(a) of the Second Amendment will survive such termination of this Agreement."

(vii) Section 7.4 of the Agreement (captioned "Consequences of Expiration or Termination") is amended and restated as follows:

"7.4 Consequences of Expiration or Termination. Expiration or termination of this Agreement will not relieve the parties of any obligation accruing prior to such expiration or termination. Except as otherwise provided in Section 7.3 of the Agreement in the case of a termination pursuant to its terms, and notwithstanding any other provision of this Agreement to the contrary, only the provisions of Sections 4.3, 4.4, 4.5, 4.6, 4.7, 7.4, and 7.5 of the Agreement, and Articles 1, 5, 6 (to the extent applicable), 8 and 9 of the Agreement and Sections 1, 4(b)(ii), and 18(a) of the Second Amendment will survive expiration or termination of this Agreement."

(viii) The second sentence of Section 7.5 of the Agreement is amended and restated as follows: "The parties agree that the Foundation, to the extent it receives a license pursuant to Section 6.1(c) of the Agreement or Second Amendment Section 3 or 13, will retain and may fully exercise all of its rights and elections under the U.S. Bankruptcy Code."

(ix) Section 9.7 of the Agreement (captioned "Notices") is amended and restated as follows:

"9.7 Notices. All notices and other communications provided for hereunder shall be in writing and shall be mailed by first-class, registered or certified mail, postage paid, or delivered personally, by overnight delivery service or by facsimile, with confirmation of receipt, addressed as follows:

"If to Foundation:

"Spinal Muscular Atrophy Foundation
"888 Seventh Avenue, Suite 400
"New York, NY 10019
"Fax: (212) 247-3079
"Attention: Ms. Cynthia Joyce, Executive Director

"With a copy to:

"Cooley Godward Kronish LLP
"4401 Eastgate Mall
"San Diego, CA 92121
"Fax: (858) 550-6420
"Attention: Matthew Browne, Esq.

"If to Company:

"PTC Therapeutics, Inc.
"100 Corporate Court
"South Plainfield, NJ 07080-2449
"Fax: 908-222-1128
"Attention: Legal Department

"With an email copy to: legal@ptcbio.com

"Either party may by like notice specify or change an address to which notices and communications shall thereafter be sent. Notices sent by facsimile shall be effective upon confirmation of receipt, notices sent by mail or overnight delivery service shall be effective upon receipt, and notices given personally shall be effective when delivered."

19. No Other Modifications. In all other respects, the terms and conditions of the Agreement shall remain unchanged and in full force and effect. In the event of any conflict between the terms of this Second Amendment and the terms of the Agreement or the First Amendment, the terms of this Second Amendment shall govern. For clarity, any cross-references to Agreement Sections refer to those Agreement Sections as amended by this Second Amendment.

IN WITNESS WHEREOF, the parties have executed this Second Amendment by their duly authorized officers as of the date set forth above.

PTC THERAPEUTICS, INC.	**SPINAL MUSCULAR ATROPHY FOUNDATION**
/s/ Stuart Peltz	/s/ Florence A. Eng
By: Stuart Peltz	By: Florence A. Eng
Title: President & CEO	Title: President

EXHIBIT SA-1

Research Plan for DC Research

[See following for Research Plan and related budget]

FTE Rates

FTE Category	FTE Rate (Annual)
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

DC Research Plan
Exhibit SA-1

[**]

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of seven pages were omitted. [**]

Note: This draft budget is based on slides presented to SMAF in November 2008 that contemplated funding by SMAF commencing in December 2008. The dates and numbers in this draft will need to be adjusted depending on the date we actually sign the contract.

DC Research Budget

Draft of December 31, 2008

Ours is a New Way of Looking

MONTHS 1-12: FTES AND EXTERNAL EXPENSES TO BE FUNDED BY SMAF

Employee Type	12/08	1/09	2/09	3/09	4/09	5/09	6/09	7/09	8/09	9/09	10/09	11/09	TOTAL
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTEs	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTE Cost ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
External Spend ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Grand Total - SMA ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

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MONTHS 13-24: FTES AND EXTERNAL EXPENSES TO BE FUNDED BY SMAF

Employee Type	12/09	1/10	2/10	3/10	4/10	5/10	6/10	7/10	8/10	9/10	10/10	11/10	TOTAL
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTEs	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTE Cost ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
External Spend ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Grand Total – SMA ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

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MONTHS 1-12: FTES AND EXTERNAL EXPENSES TO BE FUNDED BY PTC

Employee Type	12/08	1/09	2/09	3/09	4/09	5/09	6/09	7/09	8/09	9/09	10/09	11/09	TOTAL
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTEs	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

Total FTE Cost ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
External Spend ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Grand Total - PTC ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

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MONTHS 13-24: FTES AND EXTERNAL EXPENSES TO BE FUNDED BY PTC

Employee Type	12/09	1/10	2/10	3/10	4/10	5/10	6/10	7/10	8/10	9/10	10/10	11/10	TOTAL
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTEs	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total FTE Cost ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
External Spend ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Grand Total - PTC ($000s)	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

SMA PROJECT BUDGET SUMMARY

	Gap Period July 2008 -Nov 2008	Year One Dec 2008 - Nov 2009	Year Two Dec 2009 - Nov 2010	Total
SMA Request	[**]	[**]	[**]	[**]
PTC Share	[**]	[**]	[**]	[**]
Total Investment	[**]	[**]	[**]	[**]

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EXHIBIT SA-2

Format for DC Research Reports

[See following]

Project: XYZ

Date:
Period Covered:
Project Objective:

SMAF Logo

Major Accomplishments

Key Issues - Plans to Address Them

Next Major Milestones and Dates

Critical Decision-Enabling Activities and Timelines

EXHIBIT SA-3

Product Profile Format for other SMA Efforts

[See following]

Exhibit SA-3: Lead Profile Status Report (Unfunded)

Attributes	Other Effort(1)
Potency ($EC_{1.5X}$)	
Increase SMN protein level in cells (fold)	
Target selectivity	
Selectivity Index ($CC_{50}/EC_{1.5X}$)	
Metabolism (%loss in 1hr)	
Route of administration	
PK (e.g. exposure)	
hERG inhibition (% at 5 μM)	
P450 inhibition (IC_{50})	
Increase SMN expression in a mouse model	

(1) Effort by PTC in SMA field not covered under the collaboration agreement

EXHIBIT SA-4A

Development Deadline Document

This Development Deadline Document is an Exhibit to the Sponsored Research Agreement, as amended (the "Agreement"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. (the "Company"), and describes deadlines for Development as further specified in that Agreement. Capitalized terms used but not otherwise defined in this Development Deadline Document shall have the meanings set forth in the Agreement.

Activity/Milestone	Deadline (months)
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

[**]

EXHIBIT SA-4B

Company's Anticipated Development Activities and Goals

This Exhibit SA-4B to the Sponsored Research Agreement, as amended (the "Agreement"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. (the "Company"), describes the Company's expectations, as of the Second Amendment Effective Date, of the activities required to obtain Regulatory Approval and its goal timelines for completing such activities. Capitalized terms used but not otherwise defined in this Exhibit SA-4B shall have the meanings set forth in the Agreement.

Activity/Milestone	Goal (months)
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

[**]

EXHIBIT SA-5

Foundation Representatives with Access to Shared Electronic Collaboration Space

[**]

EXHIBIT SA-6

Foundation Representatives with Access to Sensitive Data in Shared Electronic Collaboration Space

EXHIBIT SA-7

Form of CDA for Foundation Representatives

[See following]

CONFIDENTIALITY AGREEMENT

This Agreement is made as of , 20 , by and between PTC Therapeutics, Inc., having an address of 100 Corporate Court, Middlesex Business Center, South Plainfield, NJ, 07080 (the "Company"), and ("Recipient"), a designated representative of the Spinal Muscular Atrophy Foundation ("Foundation") pursuant to that certain Sponsored Research Agreement dated as of June 1st, 2006, as amended (the "SRA").

1. Background. The Recipient will serve as the designated representative of Foundation pursuant to Section of the SRA.

2. Proprietary Information. As used in this Agreement, the term "Proprietary Information" shall mean all confidential or proprietary information or materials of the Company, whether disclosed in writing, orally, or visually, including, without limitation, technical information, including inventions, methods, plans, processes, specifications, characteristics, chemical structures, raw data, know-how, experience, and trade secrets; developmental, marketing, sales, operating, performance, and cost information; and all record bearing media containing or disclosing the foregoing information, including business plans, patents and patent applications, grant applications, notes, and memoranda, or drafts of any of the foregoing, whether in writing or presented, stored or maintained in or by electronic, magnetic, or other means.

3. Disclosure of Proprietary Information. The Recipient shall hold in confidence, and shall not disclose to any person, any Proprietary Information disclosed to Recipient by the Company or the Foundation except as specifically permitted herein. The Recipient shall use such Proprietary Information only to accomplish the purposes of the SRA and shall not use or exploit such Proprietary Information for his own benefit or the benefit of another (except for the benefit of the Foundation as contemplated by the SRA) without the prior written consent of the Company. The Recipient may disclose Proprietary Information received by him under this Agreement only (i) to the Foundation and its Affiliates subject to the obligations of confidentiality specified in the SRA; or (ii) to those other representatives of Foundation who have a need to know such Proprietary Information in the course of the performance of their duties and who are bound by written agreement to protect the confidentiality of such Proprietary Information in accordance with terms set forth in the SRA.

4. Limitation on Obligations. The obligations of the Recipient specified in Section 3 above shall not apply, and the Recipient shall have no further obligations hereunder, with respect to any Proprietary Information to the extent that such Proprietary Information;

(a) is generally known to the public at the time of disclosure or becomes generally known through no wrongful act on the part of the Recipient;

(b) is in the Recipient's possession at the time of disclosure to Recipient other than as a result of prior disclosure by the Company or Foundation or a breach of any legal obligation by Recipient or third party;

(c) is in the Foundation's possession at the time of disclosure to Foundation other than as a result of prior disclosure by the Company or a breach of any legal obligation by Foundation or third party;

(d) becomes known to the Recipient through disclosure by a source other than the Company or Foundation, provided that such source has no duty of confidentiality to the

Company, whether direct or indirect, with respect to such Proprietary Information and has the legal right to disclose such Proprietary Information;

(e) is independently developed by the Recipient or Foundation without reference to or reliance upon the Proprietary Information, as can be documented by written records; or

(f) is required to be disclosed by the Recipient or Foundation to comply with applicable laws or governmental regulations, provided that the Recipient provides prior written notice of such disclosure to the Company and takes reasonable and lawful actions to avoid and/or minimize the extent of such disclosure.

5. Ownership of Proprietary Information. The Recipient agrees that the Company is and shall remain the exclusive owner of the Proprietary Information and all patent, copyright, trade secret, trademark and other intellectual property rights therein. No license or conveyance of any such rights to the Recipient is granted or implied under this Agreement.

6. Return of Documents. The Recipient shall, upon the request of the Company, turn over to the Foundation, or with the Foundation's prior written permission, return to the Company, all drawings, documents, materials and other tangible manifestations of the Proprietary Information received by the Recipient pursuant to this Agreement (and all copies and reproductions thereof) except that one copy of each may be retained by Recipient for the purposes of assuring compliance with the terms of this Agreement.

7. Term. The Recipient's obligations with respect to each item of Proprietary/Information shall extend until [**] years from the end of the Term of the SRA; provided, however, that with respect to trade secret information, Recipient's obligations shall further extend until such information no longer constitutes a trade secret of Company under applicable law.

8. Miscellaneous.

(a) The Company acknowledges that the Recipient is a representative of the Foundation and that Company has the confidentiality obligations set forth in Section 5.1 of the SRA with respect to any confidential or proprietary information of Foundation disclosed to Company by the Recipient.

(b) This Agreement supersedes all prior agreements, written or oral, between the Company and the Recipient relating to the subject matter of this Agreement. This Agreement may not be modified, changed or discharged, in whole or in part, except by an agreement in writing signed by the Company and the Recipient.

(c) This Agreement will be binding upon and inure to the benefit of the parties hereto and their respective heirs, successors and assigns.

(d) This Agreement shall be construed and interpreted in accordance with the laws of the State of New York, without giving effect to conflict of laws provisions.

(e) The provisions of this Agreement are necessary for the protection of the business and goodwill of the parties and are considered by the parties to be reasonable for such purpose. The Recipient agrees that any breach of this Agreement will cause the Company substantial and irreparable harm and, therefore, in the event of any such breach, in addition to other remedies which may be available, the Company shall have the right to seek specific performance and other injunctive and equitable relief.

IN WITNESS WHEREOF, the parties hereto have caused this Agreement to be executed by their duly authorized representatives as of the date first above written.

PTC THERAPEUTICS, INC.

_____

Name:
Title:

RECIPIENT:

_____

Name:
Title:

Institutional Affiliation/Employer:

EXHIBIT SA-8

Foundation initial JSC and Joint Team Members

Foundation initial JSC members:

[**]

Foundation initial Joint Team members:

[**]

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AMENDMENT No. 3 TO SPONSORED RESEARCH AGREEMENT

This third amendment ("Third Amendment") to the Sponsored Research Agreement is effective as of the 1st day of January, 2011 (the "Third Amendment Effective Date"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. (the "Company"), with reference to the following facts and circumstances.

WHEREAS Foundation and Company are parties to that certain Sponsored Research Agreement (the "Agreement") dated as of June 1st, 2006, as amended by the First Amendment on October 12th, 2007 and by the Second Amendment on May 1, 2009 (the "Second Amendment");

WHEREAS, the parties desire to further amend the Agreement in connection with continued research, beyond the [**] specified in the Second Amendment, on small molecule therapeutics for SMA;

NOW THEREFORE, in consideration of the premises and mutual covenants contained in this Third Amendment, the parties agree as follows:

1. Continuing Research.

(a) By letter dated [**], Foundation called a special meeting of the JSC pursuant to Second Amendment Section 2(g) to address the Cost/Timeline Issue that a [**] by the [**] of [**] and that the [**] of $[**] before a [**] would be [**]. As contemplated by Second Amendment Section 2(g)(1) the JSC has agreed upon the Corrective Plan and related budget that are attached as Exhibits TA-1 and TA-2, respectively. Such Corrective Plan and related budget constitutes an amendment of the Research Plan and related budget. Company shall conduct the DC Research in accordance with the Agreement, as amended.

(b) In connection with adoption of the Corrective Plan, the JSC has also agreed upon the DC Criteria attached as Exhibit TA-3 and the parties have agreed to extend the DC Timeline Goal to [**] and the Research Term until the earliest of (i) the date upon which the JSC first designates a Development Candidate, (ii) [**] or (iii) the effective date of any termination of the Research Term pursuant to Second Amendment Section 3. If a Development Candidate is not selected by the extended DC Timeline Goal, the parties shall have the right to call a special meeting of the JSC to address [**] in accordance with Second Amendment Section [**], including by agreeing upon [**], and if applicable thereafter, the rights specified in Section Amendment Section 2(h) and/or Second Amendment Section [**].

(c) Notwithstanding Second Amendment Section 2(d), Company shall be responsible for funding one hundred percent (100%) of the total overall cost of all DC Research performed on or after the Third Amendment Effective Date; provided, however, that Company shall have the ability to set its own budgets with respect to the conduct of the research after [**] so long as the Company's obligations under Section 2.5 of the Agreement (captioned "Performance Standards") are met. Company acknowledges and agrees that Foundation has paid all amounts due to Company pursuant to Second Amendment Section 2(d) and does not have any further

obligation to reimburse Company for any amounts incurred by Company, whether before or after the Third Amendment Effective Date, with respect to the DC Research. If Foundation decides in its discretion to engage [**] or other external contract research organizations ("CROs") or academic collaborators [**] to test, after the Third Amendment Effective Date, any compounds arising from the DC Research, Foundation shall be solely responsible for paying any amounts owed to [**] or such other CROs or academic collaborators in connection with such testing.

2. Governance. Second Amendment Section 5(d) is amended and restated in its entirety as follows:

"(d) Meetings and Decision-Making by the JSC — Before Proof-of-Concept. During the Research Term and through achievement of Proof-of-Concept, the JSC shall meet periodically as needed, but in no event less than [**], in person (with the location to be at Foundation's offices in New York City unless otherwise agreed by the Parties) or by teleconference or other electronic means as mutually agreed, to discuss matters within its jurisdiction. In addition, the JSC may agree to hold special meetings at any time on reasonable notice given by the chairperson or the secretary to the other members of the JSC. Unless waived by a party in writing, at least [**] JSC representatives of each party must participate in a meeting of the JSC in order for there to be a quorum at such meeting. The members of the JSC shall seek to make all determinations to be made by them unanimously following full discussion thereof (with each party's representatives having, collectively, one (1) vote). If the JSC is unable to reach a unanimous decision on any matter within its jurisdiction, the parties' respective Chief Executive Officers shall meet in person to attempt to resolve the matter in good faith. If the parties' respective Chief Executive Officers are unable to reach agreement on a matter referred to them pursuant to the foregoing sentence within [**] days after the matter referral, then either party may by written notice to the other submit the matter to Baseball Arbitration as provided in Section 17 of this Second Amendment; provided, however, that the following matters shall not be subject to such referral to Baseball Arbitration, : (i) [**]; (ii) any [**] described in Second Amendment Section [**] as [**] to or [**]; (iii) any changes to [**] for the [**] that would require [**] than contemplated in [**]; (iv) deciding whether to pursue [**] of this Second Amendment in the event of a [**]; and (v) any disputes referred to the CEOs pursuant to Second Amendment Section [**]. Disputes not subject to referral to Baseball Arbitration pursuant clauses (i) through (v) of the preceding sentence shall be resolved as follows: any dispute arising in

the JSC with respect to clause (v) shall be decided by [**], and any disputes arising in the JSC with respect to clauses (i) through (iv) may only be resolved by mutual agreement of the parties."

3. Partnering Activities.

Second Amendment Section 10(c) is amended and restated in its entirety as follows:

"(c) If Company determines to actively pursue Collaboration Activities, whether at its own initiative or in response to inquiries from Third Parties, Company will first seek input from Foundation through a mutually-agreed team of Foundation representatives

(the "Foundation Partnering Team") on the nature, scope, and potential terms of a transaction arising in connection with such Collaboration Activities, as well as a rank-ordered summary list of preferred potential counterparties to such transaction. To the extent prepared by Company rather than received by Company from a potential counterparty, Company shall also provide the Foundation Partnering Team with an opportunity to review a draft term sheet and related materials in support of its proposed Collaboration Activities. The Foundation shall collect input from the Foundation Partnering Team on Company's overall approach to Collaboration Activities, as well as specific input on any term sheet or related materials provided to the Foundation Partnering Team, and shall promptly provide such input to Company. The initial mutually-agreed members of the Foundation Partnering Team are set forth on Exhibit TA-4. The Foundation may replace the outside counsel member of the Foundation Partnering Team with an alternative outside counsel chosen by the Foundation; such replacement will be effective upon notice to Company. The Foundation may replace any other member of the Foundation Partnering Team with an alternative individual chosen by the Foundation; such replacement will be effective upon PTC's written consent, which will not be unreasonably withheld or delayed."

Second Amendment Section 10(e) is amended and restated in its entirety as follows:

"(e) Prior to Company entering into a definitive written agreement with any Third Party in connection with Collaboration Activities, Company shall seek the review and approval of the Foundation by providing the members of the Foundation Partnering Team with a proposed final draft of the definitive written agreement, a summary (which may be oral or written) of the proposed transaction, including an overview of any items or terms subject to finalization in the draft provided, and the timely opportunity (which may include one or more in-person meetings) to discuss such draft and summary and answer the Foundation's questions with respect thereto. If required by the Company's confidentiality agreement with the potential counterparty, Company shall have the right to redact financial terms from such proposed final draft of the definitive written agreement. As promptly as reasonably possible, but in no event later then [**] business days following receipt by the Foundation Partnering Team members of such proposed final draft of the definitive written agreement and summary, Foundation shall either approve or deny such proposed transaction; *provided, however*, that if Company has otherwise complied with requirements of this Second Amendment Section 10, Foundation shall only be entitled to deny such proposed transaction if it agrees either (i) to fund [**] percent ([**]%) of ongoing Development and commercialization costs for the applicable Development Candidate(s) or Product(s), or (ii) [**] or [**] and any related rights pursuant to [**]; and *provided further*, that failure of Foundation to communicate its approval or denial of a transaction pursuant to Second Amendment Section 10(e) shall entitle PTC to treat the proposed transaction as approved by Foundation. If the Foundation denies approval in accordance with this Second Amendment Section 10(e), Company shall not enter into such proposed definitive written agreement, but shall have the right to continue the applicable negotiations consistent with

this Second Amendment Section 10 for the purposes of achieving a form of such definitive written agreement acceptable to Foundation."

4. No Other Modifications. In all other respects, the terms and conditions of the Agreement shall remain unchanged and in full force and effect. In the event of any conflict between the terms of this Third Amendment and the terms of the Agreement, the First Amendment, or the Second Amendment, the terms of this Third Amendment shall govern. For clarity, any cross-references to Agreement Sections refer to those Agreement Sections as amended by this Third Amendment.

IN WITNESS WHEREOF, the parties have executed this Third Amendment by their duly authorized officers as of the date set forth above.

PTC THERAPEUTICS, INC.	**SPINAL MUSCULAR ATROPHY FOUNDATION**
/s/ Mark E. Boulding	/s/ Florence (Loren) Eng
By: Mark E. Boulding	By: Florence (Loren) Eng
Title: SVP & General Counsel	Title: President

EXHIBIT TA-1

Corrective Plan

PTC and SMAF are proposing this amended Research Plan and related budget as a Corrective Plan to continue their existing collaboration directed towards the discovery of compounds with the potential to treat spinal muscular atrophy. [**]

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of six pages were omitted. [**]

Summary

[**].

Goal of the research proposal:

[**].

EXHIBIT TA-2

Budget for Corrective Plan

Employee Type	Jan-11	Feb-11	Mar-11	Apr-11	May-11	Jun-11	Cost	Total FTEs
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total	[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
	[**]	[**]	[**]	[**]	[**]	[**]	[**]	

External Spend (PTC)	Jan-11	Feb-11	Mar-11	Apr-11	May-11	Jun-11	Cost
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]

[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
[**]	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total	[**]	[**]	[**]	[**]	[**]	[**]	[**]
External Spend (PGI)	[**]	[**]	[**]	[**]	[**]	[**]	[**]
Total	[**]	[**]	[**]	[**]	[**]	[**]	[**]

			Cost Assumptions	
[**]		[**]	**Employee Type**	**FTE Rate**
[**]		[**]	[**]	[**]
[**]	[**]		[**]	[**]
[**]	[**]		[**]	[**]
[**]	[**]		[**]	[**]
[**]	[**]		[**]	[**]
[**]	[**]		[**]	[**]

SMA FOUNDATION **CONFIDENTIAL -Slide 1** **PTC Therapeutics**

EXHIBIT TA-3

DC Criteria

Activity	Goals	Assay Description	Notes

Confidential Materials omitted and filed separately with the Securities and Exchange Commission. A total of ten pages were omitted.

[**]

EXHIBIT TA-4

Foundation Partnering Team

Name	Relationship to Foundation
[**]	[**]
[**]	[**]
[**]	[**]
[**]	[**]

Execution version

AMENDMENT No. 4 TO SPONSORED RESEARCH AGREEMENT

This Fourth Amendment ("Fourth Amendment") to the Sponsored Research Agreement is effective as of the 22 day of November, 2011 (the "Fourth Amendment Effective Date"), by and between Spinal Muscular Atrophy Foundation (the "Foundation") and PTC Therapeutics, Inc. (the "Company"), with reference to the following facts and circumstances.

WHEREAS Foundation and Company are parties to that certain Sponsored Research Agreement dated as of June 1st, 2006, as amended by the First Amendment on October 12th, 2007, by the Second Amendment on May 1, 2009 (the "Second Amendment"), and by the Third Amendment on January 1, 2011 (as so amended, the "Agreement");

WHEREAS, the parties desire to further amend the Agreement to extend the DC Timeline Goal and the Research Term; and

WHEREAS, the parties have been coordinating with respect to Collaboration Activities involving a proposed License and Collaboration Agreement (the "Proposed Roche Agreement") by and among F. Hoffmann-La Roche Ltd, a Swiss corporation with an office and place of business at Grenzacherstrasse 124, 4070 Basel, Switzerland ("Roche Basel") and Hoffmann-La Roche Inc., a New Jersey corporation with an office and place of business at 340 Kingsland Street, Nutley, New Jersey 07110, U.S.A. ("Roche Nutley"; Roche Basel and Roche Nutley together referred to as "Roche") on the first hand, the Company on the second hand and (solely with respect to the Foundation Provisions (as defined in the Proposed Roche Agreement)) the Foundation on the third hand, which Proposed Roche Agreement is expected to be finalized in the near future;

NOW THEREFORE, in consideration of the premises and mutual covenants contained in this Fourth Amendment, the parties agree as follows:

1. Extension. The parties hereby agreed to extend the DC Timeline Goal to [**] and the Research Term until the earliest of (i) the date upon which the JSC first designates a Development Candidate, (ii) [**] or (iii) the effective date of any termination of the Research Term pursuant to Second Amendment Section 3. If a Development Candidate is not selected by the extended DC Timeline Goal, the parties shall have the right to call a special meeting of the JSC to address [**] in accordance with Second Amendment Section [**], including by agreeing upon [**], and if applicable thereafter, the rights specified in Section Amendment Section [**] and/or Second Amendment Section [**].

2. SMAF Funding Amount. As of the Fourth Amendment Effective Date, the SMAF Funding Amount shall be $13,120,140.83.

3. Proposed Roche Agreement. With respect to the Proposed Roche Agreement, the Company and the Foundation agree as follows: (a) the Foundation hereby waives the requirement for an in-person meeting set forth in Second Amendment Section 10(d)(iii) with respect to the Proposed Roche Agreement, (b) the Foundation approves the Proposed Roche

Agreement pursuant to Second Amendment Section 10(e), and (c) the effectiveness of this Fourth Amendment Section 3 shall be contingent upon the execution by the Company, Foundation and Roche of the definitive final version of the Proposed Roche Agreement.

4. No Other Modifications. In all other respects, the terms and conditions of the Agreement shall remain unchanged and in full force and effect. In the event of any conflict between the terms of this Fourth Amendment and the terms of the Agreement, the terms of this Fourth Amendment shall govern. For clarity, any cross-references to Agreement Sections refer to those Agreement Sections as amended by this Fourth Amendment.

IN WITNESS WHEREOF, the parties have executed this Fourth Amendment by their duly authorized officers as of the date set forth above.

PTC THERAPEUTICS, INC.	**SPINAL MUSCULAR ATROPHY FOUNDATION**
/s/ Stuart Peltz	/s/ Florence Eng (Loren)

By:	Stuart Peltz	By:	Florence Eng (Loren)
Title:	President and CEO	Title:	President