Investigator Initiated Study (IIS)
Proposal Submission Worksheet

At PTC Therapeutics, we are dedicated to transforming the lives of patients and their families. As part of our commitment, we provide support to independent Investigator Initiated Studies (IIS) that align with our portfolio and research interests. These studies offer us valuable opportunities to deepen our understanding of rare diseases and potentially improve patients' lives.

What is an IIS?

Investigator Initiated Studies are unsolicited, independent research studies where the investigator independently conceives the research idea, develops the study protocol, and serves as the sponsor. PTC provides support in the form of financial support and/or drug. Support of a study in no way implies any obligation toward or is in any way connected to the recommendation or prescribing of PTC products.

All IIS requests are reviewed by the PTC review committee, which comprises medical and scientific personnel. Proposals are evaluated based on scientific merit, alignment with our research interests, and available funding. A complete IIS proposal should be submitted to our portal. If the proposal is approved, investigators will be asked to submit a full protocol for further evaluation within 90 days after approval of the proposal.

Submission of a proposal does not guarantee approval. Financial and/or product support is contingent upon the full execution of a research agreement by both parties. Researchers are invited to submit their concept proposals via our submission portal after completing below registration to our portal.

Submission Process

After creating an account through our website https://www.ptcbio.com/grants-and-donations/investigator-initiated-studies, you can expect to receive an email from "PTC Encompass Admin", with login credentials. Please monitor the email you registered with closely, if the email has not been received, consider checking your email's spam folder.

Once the login credentials have been received, navigate to the Encompass website (https://ptcbio.appiancloud.com/suite/sites/iis-cu) and log in with the provided username and password. This portal is utilized to help facilitate the IIS process.

Once you have logged into Encompass, you will be able to submit your IIS request via the "Create a New IIS/ACTS Request" button on the homepage. You can use Encompass to complete key activities related to your IIS request, including:

- Proposal Submission
- Protocol Submission
- Study Tracking – including milestone submission, invoicing, tracking other study updates
- Study Closure

For additional guidance on how to submit and view requests, please contact PTCIIS@ptcbio.com.

IIS Proposal Worksheet

Study Contact Information	
Investigator-Sponsor:	
Institution:	
Specialty:	
Address:	
City:	

Submission Date:	
Telephone:	
E-mail:	
State:	
Postal Code:	
Country:	
Other:	

Other Investigators:	
PTC Therapeutics Contact:	

Study Proposal	
Study Title:	
Relevant Disease State(s):	☐ **Phenylketonuria (PKU)** ☐ **Familial Chylomicronemia Syndrome (FCS)** ☐ **Hereditary Transthyretin Amyloidosis (hATTR)** ☐ **Familial Partial Lipodystrophy (FPL)** ☐ **Other _____**
Scientific Basis/Rationale:	
Site Information	☐ **Single Site** ☐ **Multi-site, Number of sites: _____**
Country Information:	☐ **Single Country** ☐ **Multi-Country, List Countries: _____**
IRB or Ethics Committee Approval	☐ **Required** ☐ **Exempt** ☐ **Unknown**

Study Design:	☐ **Interventional** ☐ **Non-interventional**	☐ **Prospective** ☐ **Retrospective** ☐ **Ambispective**
Study Design, *please explain:*		

Study Proposal	
Hypothesis:	
Study Objectives	**Primary:**
	Secondary:
	Exploratory (if applicable):
Study Endpoints	**Primary:**
	Secondary:
	Exploratory (if applicable):

Subject Selection	
Study Population:	
Inclusion Criteria:	
Exclusion Criteria:	

Study Synopsis / Abstract:	
Sample Size & Justification:	
Estimated Study Start Date:	
Estimated Study End Date:	
Predicted Study Milestones:	

In addition to final study report, select the predicted publication plans of study findings (please indicate all that apply)

• **Publication** ☐ • **Poster** ☐ • **Abstract** ☐ • **Peer Review** ☐ • **White Paper** ☐	• **Journal Article** ☐ • **Number of Tested Samples** ☐ • **Interim Report** ☐ • **Final Report** ☐ • **Other:** ☐ _____

Plans for Publication:
Please describe plans for abstracts, publications, congress, etc.

Conflict of Interest with any PTC Employee?	☐ **Yes** *(Please explain)* _____ ☐ **No**

Funding			
Support Requested:	☐ **Funding** ☐ **Drug** ☐ **Funding & Drug** ☐ **Other**		
Estimated total study budget:		**Currency:**	
Amount of PTC Therapeutics funding requested:		**Currency:**	
Is support being requested from other entities?	☐ **Yes** *(please specify)* _____ ☐ **No**		

Proposed Budget details: *Provide a detailed budget breakdown (e.g. Personnel, Materials, etc.) with the corresponding cost & date. Please complete the following chart, using the example below as a reference.*
Example: • *Protocol Submission/EC Approval - $XXX* • *Study Initiation - $XXX* • *Patient testing - $XXX* • *Study Completion – May 1st, 2023 - $XXX*
Comment on institution's capacity for conducting study: *i.e. facilities, staffing, personnel, etc.*
Summary of Investigator relevant studies and research: *Please include a current CV with submission*
Additional Comments:

Amendment/Extension (Only if applicable)
Is this study being conducted as an Extension/Amendment of a past or current study? ☐ **Yes –** *If yes, please answer the rest of the questions in this section* ☐ **No –** *If no, please move on to the next section (Funding)*
a. Title and description of associated study:
b. What were the outcomes of the associated study?
c. Reason for pursuing extension/amendment?
d. Have previous study milestones been met? Why or why not?
e. Reason for requesting additional funding or change in budget:
f. Reason for study duration/extension: