Investigator Initiated Study (IIS)
Proposal Submission Worksheet

PTC Therapeutics is committed to helping the lives of patients and families. Part of this commitment includes providing support to independent Investigator Initiated Studies (IIS). The research derived from these studies provides opportunities to address important medical and scientific questions related to PTC compounds and therapeutic areas of interest in rare diseases.

What is IIS?

Investigator Initiated Studies (IIS) are unsolicited studies in which the investigator conceives the research, develops the protocol, and serves as the sponsor. PTC provides support in the form of a medical research grant. 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 will be submitted to the PTC review committee comprised of medical and scientific personnel for consideration. Independent requests must have clear objectives and be in alignment with areas of interest to PTC. The committee evaluates based on set criteria and evaluation process. A complete IIS proposal should be submitted 60 days prior to the program start date.

What to Expect After Submission

After submission of your IIS Request, an account will be created for you in our Encompass IIS Application Portal. This portal is utilized to help facilitate the IIS process. 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

After submission, 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.

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. For additional guidance on how to submit and view requests, please refer to the user guide on the PTC IIS homepage, or by contacting PTCIIS@ptcbio.com.

Study Contact Information	
Investigator-Sponsor:	
Institution:	
Specialty:	
Address:	
Address 2:	
City:	

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

Other Investigators:	
PTC Therapeutics Contact:	

Study Proposal	
Study Title:	
Relevant Disease State(s):	☐ **AADC Deficiency (AADCd)** ☐ **Duchenne Muscular Dystrophy (DMD)** ☐ **Familial Chylomicronemia Syndrome (FCS)** ☐ **Hereditary Transthyretin Amyloidosis (hATTR)** ☐ **Familial Partial Lipodystrophy (FPL)** ☐ **Other _____**
Areas of Interest:	☐ **Epidemiology** ☐ **Natural History** ☐ **Diagnosis & Diagnostic Testing** ☐ **Real-World Evidence** ☐ **Other _____**
Scientific Basis/Rationale:	
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:*		
Site Information	☐ **Single Site** ☐ **Multi-site, Number of sites: _____**	
CentoCard use required?	☐ **Yes –** *If yes, please answer the next question* ☐ **No –** *If no, please skip the next question*	
If CentoCards are required, number of tests expected:		
Conflict of Interest with any PTC Employee?	☐ **Yes** *(Please explain)* **_____** ☐ **No**	

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

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

Predicted Study Milestones:

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

Subject Selection
Study Population:
Inclusion Criteria:
Exclusion Criteria:

Amendment/Extension
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:

Funding			
Support Requested:	☐ **Funding** ☐ **Resources** ☐ **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 detail aligned with key milestones:

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 - Mar 1st, 2022 – $XXX*
- *Study Initiation – May 1st, 2022 - $XXX*
- *Study Milestones (# Patients Enrolled) – June 1st, 2022 - $XXX*
- *Study Completion – May 1st, 2023 - $XXX*

Milestone Title	***Date***	***Estimated Cost***
•		
•		
•		

Please note, the total cost listed here should equal to the "Amount of PTC Funding Requested" above.

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

Plans for Publication:

Please describe plans for abstracts, publications, congress, etc.

Additional Comments: