

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 for PTC to better understand rare diseases and potentially improve patients' lives.

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.

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.

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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):	
Scientific Basis/Rationale:	
Country Information:	<input type="checkbox"/> Single Country <input type="checkbox"/> Multi-Country, List Countries: _____
IRB or Ethics Committee Approval	<input type="checkbox"/> Required <input type="checkbox"/> Exempt <input type="checkbox"/> Unknown
Study Design:	<input type="checkbox"/> Interventional <input type="checkbox"/> Non-interventional <input type="checkbox"/> Prospective <input type="checkbox"/> Retrospective <input type="checkbox"/> Ambispective
Site Information	<input type="checkbox"/> Single Site <input type="checkbox"/> Multi-site, Number of sites: _____
CentoCard use required?	<input type="checkbox"/> Yes <input type="checkbox"/> No
Conflict of Interest with any PTC Employee?	<input type="checkbox"/> Yes (Please explain) _____ <input type="checkbox"/> 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:	
<ul style="list-style-type: none"> • Publication <input type="checkbox"/> • Poster <input type="checkbox"/> • Abstract <input type="checkbox"/> • Peer Review <input type="checkbox"/> • White Paper <input type="checkbox"/> 	<ul style="list-style-type: none"> • Journal Article <input type="checkbox"/> • Number of Tested Samples <input type="checkbox"/> • Interim Report <input type="checkbox"/> • Final Report <input type="checkbox"/> • Other: <input type="checkbox"/> _____

Subject Selection
Study Population:
Inclusion Criteria:
Exclusion Criteria:

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Amendment/Extension	
<p>Is this study being conducted as an Extension/Amendment of a past or current study?</p> <p><input type="checkbox"/> Yes – <i>If yes, please answer the rest of the questions in this section</i></p> <p><input type="checkbox"/> No – <i>If no, please move on to the next section (Funding)</i></p>	
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:	<input type="checkbox"/> Funding	<input type="checkbox"/> Resources	<input type="checkbox"/> Drug <input type="checkbox"/> Other
Estimated total study budget:		Currency:	
Amount of PTC Therapeutics funding requested:		Currency:	
Is support being requested from other entities?	<input type="checkbox"/> Yes (please specify) _____ <input type="checkbox"/> No		

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<p>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</p>																		
<p>Example:</p> <ul style="list-style-type: none"> • 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 																		
<table border="1" style="width: 100%; border-collapse: collapse; margin-top: 10px;"> <thead> <tr> <th style="text-align: center; padding: 5px;"><i>Milestone Title</i></th> <th style="text-align: center; padding: 5px;"><i>Date</i></th> <th style="text-align: center; padding: 5px;"><i>Estimated Cost</i></th> </tr> </thead> <tbody> <tr> <td style="padding: 5px;">• Protocol Submission/EC Approval –</td> <td></td> <td></td> </tr> <tr> <td style="padding: 5px;">• Study Initiation –</td> <td></td> <td></td> </tr> <tr> <td style="padding: 5px;">• Study Milestones –</td> <td></td> <td></td> </tr> <tr> <td style="padding: 5px;">• Study Completion –</td> <td></td> <td></td> </tr> <tr> <td style="padding: 5px;">• Other Milestones, please list –</td> <td></td> <td></td> </tr> </tbody> </table>	<i>Milestone Title</i>	<i>Date</i>	<i>Estimated Cost</i>	• Protocol Submission/EC Approval –			• Study Initiation –			• Study Milestones –			• Study Completion –			• Other Milestones, please list –		
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<p>Comment on institution’s capacity for conducting study: <i>i.e. facilities, staffing, personnel, etc.</i></p>																		
<p>Summary of Investigator relevant studies and research: <i>Please include a current CV with submission</i></p>																		
<p>Plans for Publication: <i>Please describe plans for abstracts, publications, congress, etc.</i></p>																		
<p>Additional Comments:</p>																		