Boston Children's Hospital

Date: Wednesday, April 29, 2020 11:59:55 AM Print Close

Title: Sample Individual Patient Expanded Access

General Information

1 * **Protocol Title:**
Sample Individual Patient Expanded Access

Maximum of 230 characters may be entered.

2 **Full Title - If protocol title exceeds the 230 characters limited from field above, enter full title here. Otherwise, leave blank.**
Sample Individual Patient Expanded Access

3 * **Provide a brief summary (in lay terms) of the research protocol.**
Brief summary

4 * **Principal Investigator (PI):** PI Test

4.1 * **To serve as a PI you must qualify under one of the following eligibility requirements. (Residents, interns, fellows and postdoctoral candidates are not permitted to be PIs). Please select the appropriate category that applies to you.**
Physicians, Dentists and Psychologists credentialed through the hospital with the BCH medical staff registrar as an active medical staff member and having an appointment of Instructor or higher at Harvard Medical School.

If Other patient services professionals:

4.1.1 **Research is part of your scope of employment responsibility and not to meet a training or degree requirement. Please explain how this research falls within the scope of your responsibilities at the hospital.**

4.1.2 **You have training and experience and confirmed clinical research competencies. Please explain your training and experience in clinical research.**

4.1.3 **Are you employed at Children's as a nurse or do you have nursing credentials through Boston Children's Hospital?**
Please note if this is checked yes, in accordance with the policies of the Nursing Department your protocol will be sent to the Nursing department for both scientific review and departmental sign off.
○ Yes ○ No

5 * **Is the person who will be primarily responsible for conducting the study at BCH different from the PI?**
○ Yes ● **No**

If YES:

5.1 **Please add the person(s) who will be primarily responsible for conducting the study.**

Name	Appointment with Children's Hospital?
There are no items to display	

6 **Has the PI, or if question #5 was YES has that person, previously served as a PI of a protocol involving interaction/intervention with human subjects at CHB?**
● **Yes** ○ No

7 * **Type Of Submission:**
- ○ New Research Activity
- ○ **New Research Activity Limited to Secondary* Use of Biological Material and Data
- ○ Establishment of Human Biological Specimen Repository/ Data Registry (only) – repositories/registries are defined as a prospective collections of specimens or data that are processed, stored, distributed to multiple investigators for use in research.
- ○ Request for Exemption
- ● **Individual Patient Expanded Access**
- ○ Humanitarian Use Device (HUD)
- ○ Reliance on Another IRB

○ Projects that lack immediate plans for involvement with human subjects, their data and/or their specimens (i.e.training grants)

*** Use this form only if:*
1) specimens/data are not identifiable or
2) specimens/data are identifiable but recorded by PI in de-identified format or meet the waiver of HIPAA authorization criteria listed below All other uses of secondary specimens/data must be submitted on a new research activity form.

** Secondary means the tissue or data will be or was collected for a primary or initial purpose other than the research (i.e data from medical records, tissue from pathology)*

Waiver of HIPAA authorization (all criteria must be met)
• The proposed use of this data/document/record/specimen presents no more than minimal risk to the privacy of individuals
•The research could not practicably be conducted without the waiver of HIPAA authorization
• The research could not practicably be conducted without access to and use of protected health information with identifiers
• Waiving HIPAA authorization will not adversely affect the subject's rights or welfare

This form may not be selected if the study involves interaction/intervention with subjects in order to obtain tissue/data specifically for this research.

8 * **Is this protocol related to child health (including perinatology, prenatal assessments, childhood antecedents of adult disease, and long-term follow up of pediatric disorders)?**

● **Yes** ○ No

9 * **Is this protocol related to cancer (primarily concerning malignancies, oncology patients, or involving use of malignant tumors)?**

○ Yes ● **No**

Note: If YES, your protocol will require review by the Dana Farber IRB instead.
For details, see: IRB Policy 3.12, 'Reliance Agreements'

10 * **Will this protocol utilize any of the services of the ETU (Experimental Therapeutics Unit)?**
Please select "No" for the following types of submission:
1. Request for Exemption
2. Projects that lack immediate plans for involvement with human subjects, their data and/or their specimens (i.e.training grants)

○ Yes ● **No**

These services include:

- *Use of space on the ETU or research space at Waltham*
- *Nursing assistance at above sites*
- *Off-site nursing and/or research coordinator services provided through ETU*
- *Specimen collection or processing, sample storage and preparation for shipping*
- *Assistance from nutritional Metabolic Phenotyping Core (preparation of research meals, analysis of food records, etc.)*
- *Use of specialist equipment located on the ETU (3DMD camera, DXA, pQCT, V-max, etc.)*

Note: If YES, your protocol will be routed for Harvard Catalyst CRC Protocol Review PRIOR to BCH IRB review. For details, see: Institutional Centers for Clinical and Translational Research (ICCTR)

11 * **Does this protocol include COVID-related research with subjects diagnosed or suspected with COVID19 that meet any of the following criteria?**
• Use of discard clinical samples (nasal swabs, blood, etc.)
• Collection of clinical samples from patients (blood, nasal swabs, sputum, urine, stool etc.)
• Collection of demographic and clinical information at time of patient encounter
• Interaction or intervention with patients (therapies, extra testing , interviews) while in the hospital (inpatient, ambulatory, emergency department)

○ Yes ● **No**

Note: Do not check "Yes" for research limited to retrospective or prospective collection of data or surveys/interviews conducted with families and patients through non inperson encounters.

Note: If "Yes" - the scientific review will be automatically routed to a newly formed SRC committee established to conduct COVID19 research reviews. In addition you are required to obtain approval by institutional representatives who have been assigned responsibility by hospital location for prioritizing multiple requests, assuring protocols meet standards for infection control, and appropriate personnel are involved.
Please contact them early during your research planning so they can provide guidance.
Please note that the processes, capabilities, and requirements differ by site.

Investigators with proposals than span different locations should discuss their research plan with all site leads:
ED: Mark Neuman, MD
ICU and ORs: Adrienne Randolph, MD
In-patient: Benji Raby, MD
Laboratory Medicine: Orah Platt, MD and Nira Pollock, MD

If you would like to request ICCTR support please contact Andy Place, MD (Chief Medical Officer) and Cindy Williams, RN MS, NE-BC (nursing)

Research Team

If the person you need to add to your protocol cannot be found using the "Add" buttons below, please send an email to CHERP Support (cherp.support@childrens.harvard.edu) requesting that the person be added to the Research Staff. CHeRP Support will need the following information:

- First Name
- Last Name
- CHID# (if applicable)
- BCH Department (if applicable)
- Email Address

1 **Research Staff - Children's Hospital Employees only:**

	Last Name	First Name	Role	Editor	CC on Correspondence	Required Training Completed	CHeRP Training	Date Modified	Date Created
View	Kuniholm	Ashley	Admin Contact	yes	yes	yes	yes	12/4/2019	12/4/2019

2 ***NOTE:*** **Accounts are no longer required for non-BCH researchers. These individuals remain under the jurisdiction of their home institution's IRB and should not be listed here. If you think there is a special circumstance, please contact your IRB Administrator.**

Research Staff - Non Children's Hospital Employees only:

Last Name	First Name	Role	Email	Required Training Completed
There are no items to display				

3 **PI:** PI Test

Completed Training Courses:

Training Program	Continuing Education Description	Training Completed	Date Created
Continuing Education	Collaborative IRB Training Initiative (CITI Continuing Education)	7/22/2018	
Continuing Education	Collaborative IRB Training Initiative (CITI Continuing Education)	7/12/2018	
Continuing Education	Continuing Education/Department Meeting	5/2/2018	
Continuing Education	Continuing Education/Department Meeting	6/13/2016	
Training Received at Another Institution		11/15/2015	
Continuing Education	Continuing Education/Department Meeting	10/26/2015	
Continuing Education	Research Protocol Case Discussions	11/15/2012	
Continuing Education	Collaborative IRB Training Initiative (CITI Continuing Education)	5/9/2012	5/9/2012
Continuing Education	Continuing Education/Department Meeting	9/30/2011	
CHeRP Training		12/19/2010	
Continuing Education	Collaborative IRB Training Initiative (CITI Continuing Education)	5/15/2009	11/8/2010
Collaborative IRB Training Initiative (CITI Behavioral)		8/2/2006	11/8/2010
Collaborative IRB Training Initiative (CITI Biomedical)		8/2/2006	11/8/2010
Collaborative IRB Training Initiative (CITI Non-Interventional)		4/11/2006	11/8/2010
Continuing Education	Collaborative IRB Training Initiative (CITI Continuing Education)	4/5/2006	11/8/2010

Title: Sample Individual Patient Expanded Access

Funding Sources

1 * **Select funding category.**

- (●) **Externally sponsored (federal, state, corporate, foundations)**
- ( ) Internally sponsored
- ( ) Externally and internally sponsored
- ( ) No sponsor
- ( ) Private Donor

1.1 If internally sponsored - select as appropriate:

- [ ] Department/ Division or Children's foundation funds
- [ ] Internal Children's Grant Award

1.2 Enter any additional information if applicable:

1.3 If the protocol does not have a sponsor, please detail how the study will be conducted without funding.

1.4 Please provide the name of the private donor.

Funding Sources - Details

1 * **List of external sponsors for this protocol.**

	Sponsor	Funding Category
View	NOVARTIS PHARMACEUTICALS CORPORATION - 1093	Corporate/Industry

Financial Disclosure

1 * **Do you or any person affiliated with the protocol have or expect to have any investment or financial relationship (examples below) with any entity that is providing funds or other support in connection with the protocol?**

( ) Yes (●) **No**

If YES:

1.1 Please select the relationships as appropriate.

- [ ] Consulting
- [ ] Payments for protocol/study design
- [ ] Protocol-related payments not included in the research agreement budget
- [ ] Stock or Options
- [ ] Honoraria
- [ ] Scientific Advisory Board Membership
- [ ] Royalties or license fees related to the protocol, or to any test article or device which will be employed in the conduct of the research under the protocol (including any royalties or license fees received through an academic institution, including Children's Hospital).
- [ ] Equipment or other laboratory support
- [ ] Other support for research unrelated to the protocol
- [ ] Support for educational or other academic or medical efforts
- [ ] Other Grants
- [ ] Other

2 * **Do you or any person affiliated with the protocol have or expect to have any proprietary interest related to the protocol, or related to any test article or device that will be employed in the protocol? Include proprietary interests that you have assigned to any entity, including any institution you have been affiliated with.**

( ) Yes (●) **No**

If YES:

2.1 Please select the proprietary interest as appropriate.

- ☐ Patent-licensed, in whole or part, to an entity providing funds for the research
- ☐ Patent-licensed, in whole or part, to another entity
- ☐ Other

3 * **Do you or any person affiliated with the protocol have or expect to have any advisory role, appointment, or employment with any entity that is providing funds or other support for the research to be conducted under the protocol?**

○ Yes ● **No**

If YES:

3.1 Please select as appropriate.

- ☐ Scientific Advisory Board Membership
- ☐ Other Advisory Role
- ☐ Officer
- ☐ Director
- ☐ Employment
- ☐ Other

4 * **Do you or any person affiliated with the protocol have or expect to have any financial interest, financial relationship, or position or advisory role with any other entity that may be affected by the research to be conducted under the protocol (e.g. competitor, customer, collaborator or commercial sponsor affiliate)? Include any entity that may be benefited or harmed, directly or indirectly.**

○ Yes ● **No**

5 * **Do you or any person affiliated with the protocol have or know of any arrangement or understanding, tentative or final, relating to any future financial interest, financial relationship, future grant, position, or advisory role either related to the protocol, or dependent on the outcome of the research under the protocol?**

○ Yes ● **No**

6 * **The IRB prohibits special incentives in connection with clinical research, including, finder's fees, referral fees, recruitment bonuses, enrollment bonuses for reaching an accrual goal, or similar types of payments. Will you or anyone else in connection with the conduct of any research under the protocol receive money, gifts or anything of monetary value that is above and beyond the actual costs of enrollment, research conduct, and reporting of results, from the sponsor or any other entity?**

○ Yes ● **No**

7 * **Is there anything not disclosed above which you believe might constitute a conflict of interest or an appearance of a conflict of interest in connection with the protocol?**

○ Yes ● **No**

8 If any of the questions above are checked "Yes", please provide the name of the individual for whom the disclosure is made and describe in further details the disclosure. This section must include a full description of the financial relationship, including but not limited to, a detailed description, as applicable, of any test article of device involved; the advisory role or appointment; the competitor, customer, collaborator; any arrangement related to the research; and so on. Please also include actual amounts of any consulting or other monies received and the time period for which it was received. This section will not be reviewed without a full disclosure.

9 Upload any other pertinent documentation.

Name	Date Last Modified	Version	Owner

There are no items to display

Individual Patient Expanded Access

1 * **Patient Name**
Patient name

2 * **Patient Medical Record Number**
medical record number

3 * **Please check one category. The physician may have received an eIND or IDE number via phone or email from the FDA in emergency situations, but the full, written submission will still need to be submitted.**

- ○ This is an individual patient request but is NOT an emergency
- ○ This is an emergency for an individual patient and is being reported to the IRB prior to initiation (whenever possible the application must be submitted prior to the emergency treatment)
- ● **This is an emergency for an individual patient and being reported to the IRB within 5 working days of initiation**

3.1 * **What is the estimated date to initiate the proposed therapy? If the emergency therapy already occured, please enter date therapy was administered.**
12/4/2019

3.2 **Please indicate which category is applicable. Please note, at least one of the two following categories must be checked if the emergency use is being reported to the IRB within 5 working days of initiation of therapy.**

- ☑ Patient is in a life threatening situation.
Life threatening means diseases or conditions where the likelihood of death is high unless the course of the disease is interrupted and a disease or conditions with a potentially fatal outcomes, where the end-point of a clinical trial is survival. The criteria for life threatening do not require the condition to be immediately life threatening or to immediately result in death. Rather, the participants must be in a life threatening situation that requires intervention before review at a convened meeting of the IRB is feasible.

- ☐ Patient is in a situation which may be subject to severe debilitation by waiting for the next IRB scheduled meeting.
Severely debilitating meaning the disease or condition may cause major irreversible morbidity.
Examples of severely debilitating conditions include blindness, loss of arm, leg, hand, or foot, loss of hearing, paralysis or stroke. To see next IRB meeting date click: here

3.2.1 **Please justify why the proposed treatment meets the criteria listed above, why there was no standard acceptable alternatives treatments and why there is not sufficient time to the IRB prior to initiation of therapy.**
Justification

4 * **Provide a brief summary of the clinical history of the patient.**
Summary of clinical history

5 * **Describe the therapy and provide the rationale for therapy.**
Description of therapy

6 * **Provide a statement on the known risks and benefits.**
Potential risks and benefits

7 * **Have previous individual patient expanded access requests involving this same treatment been submitted for other Boston Children's Hospital patients?**

○ Yes ● **No**

If YES:

7.1 **How many individual patient expanded access requests have been submitted?**

7.2 **What is the experience to date with previous individual patient expanded access requests? How are the patients doing?**

8 * **Do you anticipate that more patients will require this treatment in the future?**

○ Yes ● **No**

If YES:

8.1 **What plans are there for submission of a formal protocol for IRB review? If a protocol is already in place, please explain why this patient is not eligible for the active protocol.**

9 * **Is a drug being used?**

● **Yes** ○ No

10 * **Is a device being used?**

● **Yes** ○ No

Individual Patient Expanded Access - Drug

Drug Category

1 * **The drug is an Investigational drug.**

● **Yes** ○ No

If YES:

1.1 What is the generic name or descriptor of the product?
Generic name

1.2 What, if any, is the commercial/trade name of the product?

1.3 Who is the manufacturer of the product?
Manufacturer

1.4 Who is the supplier of the product?
Supplier

2 * **Has a Form 3926 been submitted to the FDA?**

● **Yes** ○ No

If YES:

2.1 Was box 10B checked off, indicating a request to obtain concurrence by the IRB chairperson or by a designated IRB member in order to comply with FDA's requirements for IRB review and approval?

● **Yes** ○ No

2.2 Please upload a copy of the FDA Form 3926 that was submitted

Name	Date Last Modified	Version	Owner
FDA Form 3926.pdf	12/4/2019 2:32 PM	0.01	Ashley Kuniholm

3 * **Has an individual patient expanded access IND has been granted by the FDA prior to initiation of therapy, either verbally, via email, or with a formal study may proceed letter?**

● **Yes** ○ No

If YES:

3.1 IND#
000000

3.2 Sponsor (May be drug company or investigator)
Physician name

3.3 Please upload the Study May Proceed letter or other approval documentation (i.e. email) from the FDA.

Name	Date Last Modified	Version	Owner
IND Study May Proceed.docx	12/4/2019 2:32 PM	0.01	Ashley Kuniholm

If NO:

3.4 Have you been in contact with the FDA to determine if an emergency use IND will be granted without formal submission prior to use? If so, please note that you will need to submit a written application to the FDA within 15 working days.

3.5 Provide the name and phone number of the individual contacted at the FDA to make this determination. You may also attach any written correspondence.

3.6 Upload any relevant documents.

Name	Date Last Modified	Version	Owner

There are no items to display

Individual Patient Expanded Access - Device

1 * **Device Category.**

○ This device is a Non Significant Risk device

● **This device is a Significant Risk device**

1.1 * **Have you or the sponsor obtained an IDE or submitted an IDE supplement for this expanded access request?**

● **Yes** ○ No

If YES:

1.1.1 IDE #
000000

1.1.2 Sponsor (may be company or investigator)
Sponsor

If NO:

1.1.3 If no IDE exists, have you submitted an expanded access request directly to the FDA? Please note that if this investigational device is being used in an emergency situation without prior approval by the FDA, you will need to submit a follow-up report to the FDA within 5 days.

1.1.4 Provide the name and phone number of the individual contacted at the FDA to make this determination. You may also attach any written correspondence as well.

1.1.5 Upload any relevant documents, including any the request to the FDA and approval correspondence.

Name	Date Last Modified	Version	Owner
There are no items to display			

Single Patient Emergency - Financial Considerations

1 * **Who will pay for the cost of the test article and intervention (drug or device)?**

- [x] **Sponsor/Manufacturer**
- [ ] Children's Hospital
- [ ] Patient's Insurance*
- [ ] Other

If Other:

1.1 Please describe:

2 * **Who will pay for costs associated with use of the test article and intervention (surgical procedures, added tests, hospitalization, added time in hospital, etc)?**

- [ ] Sponsor/Manufacturer
- [ ] Children's Hospital
- [x] **Patient's Insurance***
- [ ] Other

If Other:

2.1 Please describe:

** If the patient's insurance will be billed for the drug, device, or procedures related to this emergency treatment, please contact Patient Financial Services to confirm whether the insurance company will cover the costs.*

Individual Patient Expanded Access - Informed Consent

1 * **Please select one of the following:**

1.1 [x] Informed consent will be obtained from the subject, parent/guardian or legally authorized representative.

Upload a copy of the informed consent form with all the required elements.

Name	Date Last Modified	Version	Owner
Consent Form.docx	12/4/2019 2:34 PM	0.01	Ashley Kuniholm

NOTE: Your consent must use the current required format. Click here to download the template.

1.2 [ ] Informed consent cannot be obtained.

If checked, choose one of the following options:

[ ] **Option 1 - PI and a physician who is not otherwise participating in the Institutional Review Board have certified all of the following.**

Please check each box:

- [ ] The participant is confronted by a life-threatening situation necessitating the use of the test article.
- [ ] Informed consent cannot be obtained from the participant because of an inability to communicate with, or obtain legally effective consent from, the participant.
- [ ] Time is not sufficient to obtain consent from the participant's legal representative.
- [ ] There is no available alternative method of approved or generally recognized therapy that provides an equal or greater likelihood of saving the life of the participant.

[ ] **Option 2 - PI certifies that all of the following are true.**

Please check each box:

- [ ] Immediate use of the test article is, in PI's opinion, required to preserve the life of the participant.

☐ Time is not sufficient to obtain the independent determination a physician who is not otherwise participating in the Institutional Review Board.

☐ Before the use of the test article, PI certifies the following:

- The participant is confronted by a life-threatening situation necessitating the use of the test article. Informed consent cannot be obtained from the participant because of an inability to communicate with, or obtain legally effective consent from, the participant.
- Time is not sufficient to obtain consent from the participant's legal representative.
- There is available no alternative method of approved or generally recognized therapy that provides an equal or greater likelihood of saving the life of the participant.

☐ After the use of the test article, PI will obtain from a physician who is not otherwise participating in the Institutional Review Board a certification in writing within 5 working days after the use of the article of all of the following:

- The participant was confronted by a life-threatening situation necessitating the use of the test article.
- Informed consent could not be obtained from the participant because of an inability to communicate with, or obtain legally effective consent from, the participant.
- Time was not sufficient to obtain consent from the participant's legal representative.
- There was available no alternative method of approved or generally recognized therapy that provided an equal or greater likelihood of saving the life of the participant

1.2.1 Upload certification from physician.

Name	Date Last Modified	Version	Owner
There are no items to display			

3 If applicable, provide copies of any materials, protocols, investigational brochures provided by the sponsor or drug/device manufacturer or correspondence with FDA or any additional material.

Name	Date Last Modified	Version	Owner
IB.docx	12/4/2019 2:34 PM	0.01	Ashley Kuniholm

Title: Sample Individual Patient Expanded Access

Additional Documents

1 Please upload any additional documents if it is necessary.

Name	Date Last Modified	Version	Owner
There are no items to display			

Detailed Sponsor Information

1 * What is the sponsor's name?
NOVARTIS PHARMACEUTICALS CORPORATION - 1093

1.1 If your sponsor is not in the list, please select "Other" from the list and specify your sponsor below.

Note: Use a '%' to conduct a wildcard search (e.g. a '%Pharm' search will return all options with 'pharma' at any place in the name).

2 * Please select the appropriate category of funding.

○ Federal

○ State

● **Corporate/Industry**

○ External Foundation

2.1 If the category of funding is "Federal", upload the grant(s) here. (Please include the scientific part. This is a requirement for federally supported research. You need not include biosketches or financial information here, just the description of the research.)

Name	Date Last Modified	Version	Owner
There are no items to display			

3 * **What will the sponsor provide? Check all that apply:**
Drug

4 * **What is sponsor's contact name, if applicable?**
Contact name

5 * **What is sponsor's contact phone number?**
Contact phone number

6 * **What is sponsor address?**
Contact address

7 * **What is sponsor email address?**
contact@email.com

8 * **Is a Clinical Trial Agreement (CTA) required?**

- ○ Completed/Signed
- ○ Pending
- ● **Not Required**

ID: VIEW46F5DA7D2D400
Name: Detailed Sponsor Information