APPENDIX: INFORMED CONSENT TEMPLATE FOR INDIVIDUAL PATIENT EXPANDED ACCESS[1]

Disclaimer: The purpose of this informed consent template is to assist investigators with preparing an informed consent document for the treatment of a single patient with an investigational drug under the expanded access program. However, this template is not a substitute for the Federal Food, Drug, and Cosmetic Act (FD&C Act) or the Code of Federal Regulations (CFR) and does not necessarily contain all information required to ensure compliance in a given situation. Investigators are responsible for ensuring that the informed consent requirements of 21 CFR part 50 are met (21 CFR 312.305(c)(4)) unless one of the exceptions found in part 50 applies.

1. Introduction

Provide the following information:

- *The name of the disease or condition for which the investigational drug will be provided for treatment.*
- *A statement that the patient does not have any alternative Food and Drug Administration (FDA)-approved medical product (e.g., drug/biologic)[2] available to them for treatment.*
- *The name of the investigational drug/biologic.*
- *An explanation that the product is investigational, is not approved by FDA as safe and effective, and that the treatment will be considered an experimental treatment. A statement that the treatment may only proceed under FDA's expanded access program, with FDA authorization.*
- *A statement that the patient's participation in the program is voluntary and that the patient may change their decision to participate. Provide the name of the person the patient may contact in case the patient changes their decision.*
- *A statement that refusal to participate will involve no penalty or loss of benefits to which the patient is otherwise entitled and that the patient may discontinue participation at any time without penalty or loss of benefits to which the patient is otherwise entitled.*
- *A recommendation to read the form carefully and discuss with others before making any decision.*
- *The name of the staff whom the patient can contact if the patient has questions.*

[1] This template consists of instructions (in italics) to create the template and includes some example language (below the instructions) for each element. Once the template is finalized, delete the instructions from the template.

[2] In these examples, *drug* is used as a reference. In your document, use drug or biologic, as appropriate.

Examples:

- You are diagnosed with disease X.

- For your condition, there is no drug approved by the Food and Drug Administration (FDA) for use in routine medical care in the United States. **OR** The Food and Drug Administration (FDA)-approved drug or drugs available for your treatment did not work for you. **OR** You cannot tolerate the side-effects of the drug or drugs approved by the Food and Drug Administration (FDA) for treatment of your condition.

- Your doctor would like to treat you with drug Y.

- Drug Y is an investigational drug. It is *NOT* approved by FDA for the treatment of your disease. However, for your case, FDA authorized Dr. Z to treat you with the investigational drug Y under FDA's expanded access program, OR Dr. Z has requested or will request FDA's permission to treat you with the investigational drug Y under FDA's expanded access program.

- Whether or not you take this investigational drug is up to you. If you choose not to receive the investigational drug, it will not result in penalty or loss of benefits to which you are otherwise entitled.

- You can choose to take the investigational drug now but change your mind later. Tell your doctor right away about your decision if you change your mind later. It will not result in any penalty or loss of benefits to which you are otherwise entitled.

Read this document carefully. You may want to discuss your options with your doctors, family, friends, and others before deciding on whether to receive the treatment. Please ask questions about anything you do not understand. You will find a contact information table at the end of the document.

2. What are the potential benefits of receiving the treatment?

List potential benefits of the investigational drug/biologic, if any. Include a statement to reflect that the anticipated benefit may be uncertain or that the disease may worsen with the treatment.

Examples:

- There is a chance that the investigational drug Y may (1) improve . . ., (2) reduce . . ., etc. However, there is no guarantee that it will happen in your case.

- Dr. Z would like to treat you with the investigational drug because she believes that it may benefit you. However, there is no guarantee that you will benefit from this investigational treatment. It is possible that you will receive no benefit other than receiving the standard care (regularly seen by a doctor, evaluated for your condition, etc.) associated with receiving this treatment, or it could worsen your condition.

- We do not know if this investigational drug will help you. Your condition may get better, stay the same, or possibly get worse.

3. What are the potential risks of this treatment?

Provide a list of reasonably foreseeable risks or side effects of the investigational drug/biologic. Include frequency, if known. Include information on risks that are more likely to occur and those that are serious. Discuss any potential risks from the medical procedures necessary to administer the drug/biologic, if appropriate. Provide specific instructions for whom the patient should contact if experiencing serious side effects.

Examples:

- There is a risk that the investigational drug Y makes your condition worse.

- The following are serious side effects that have been reported for the investigational drug Y:
  - Serious injury to your kidneys that could lead to dialysis
  - Significant disability

- The following are side effects that are more likely to occur:
  - Vomiting
  - Diarrhea
  - Lack of appetite

- The investigational drug needs to be administered via [W] route of administration during [X procedure]. Risks of [X procedure] may include headache, pain or numbness in the legs and lower back, and bleeding into the spinal canal where the main nerve that goes down your back is located. The doctors who will perform the [X procedure] are specifically trained and very experienced in performing this procedure.

- There may be side effects of the investigational drug Y that we do not know about.
  - These effects could be immediate and short term, or your future health may be affected in ways that we currently do not understand.

- If you experience side effects listed above or any other adverse effects, contact the staff listed in the contact information table.

- In case of emergency, contact the staff listed in the contact information table or get emergency medical help immediately.

4. How long will you be treated with the investigational drug/biologic?

Describe the length of time the treatment will last (e.g., hours, days, weeks, months, years, or until a certain event), as well as long-term follow-up, if appropriate. Include number of visits or treatments as applicable.

Examples:

- You will receive the investigational drug approximately every 2 months (6–8 weeks) for up to 1 year.

- After you complete this treatment, you will still need to come to the clinic for follow-up visits for at least the next year.

5. If you do not accept this treatment, what are the other choices?

Explain that to provide an investigational drug/biologic under expanded access, the doctor should determine there is no available comparable or satisfactory alternative therapy to diagnose, monitor, or treat the disease or condition and that the doctor has made such a determination.

Examples:

- Dr. Z determined that there are no other drugs approved to treat your disease.

- There are no other drugs approved for your disease or clinical trials that you could enroll in. However, you can discuss other options with Dr. Z, such as not taking any investigational drug.

6. What are the procedures associated with the treatment?

Describe in chronological order the procedures that are necessary as part of receiving the treatment. Use a table, if needed, to organize the information. If describing every procedure will make the document too lengthy or detailed, include the information as an addendum.

Examples:

Date (chronological order)/Frequency	Description of therapy; dose, route of administration	Duration
Approximately every 2 months (i.e., 6–8 weeks)	Administer drug Y in your vein (10 mg/kg)	90 minutes

Date (chronological order)/Frequency	Procedure for assessment	Purpose
Day 1	Collect blood samples	Routine laboratory tests
Month 4	CT scan to take a picture of your X	If there is any change in the size of the tumor

7. Can your doctor stop the treatment without your permission?

Provide a list of reasons for which the doctor may stop the treatment without the patient's consent. Explain that the patient will be notified if this happens.

Examples:

In certain situations, your doctor may need to stop the investigational drug without your permission if:

- Your condition gets worse.
- The investigational drug is no longer safe for you.
- New information suggests that this investigational drug does not work.
- You become pregnant.
- New information suggests that another investigational drug is better.
- FDA tells your doctor that your treatment should be stopped. This may happen if FDA receives new information about the investigational drug that your doctor may not know because it is confidential.
- The investigational drug is no longer available from the manufacturer.

If your doctor stops your treatment, we will tell you as soon as possible.

8. What is the cost of the treatment?

Explain that the patient may incur expenses for the treatment with the investigational drug/biologic. Explain to the best of your knowledge what costs the patient is likely to need to

cover and that insurance may not cover all costs. Because the coverage of treatment with an investigational drug/biologic could be complex, it may be appropriate to recommend that the patient consult their insurer about reimbursement before initiating the treatment.

Examples:

- You or your insurance company will be charged for the treatment. You will be responsible for any costs your insurance does not cover. Contact your insurance company if you have any questions about these costs or what out-of-pocket expenses you may have.

- The [INSTITUTION] will pay for the treatment, including treatment of any side effect, adverse reaction, illness, or injury to you resulting from the treatment.

- If you receive this treatment, your insurance may not cover the cost of some of the tests and visits to see your doctor that are related to receiving this investigational drug. Contact your insurance company to learn more about the coverage if you decide to receive the treatment.

- Dr. Z's research funds will pay for some items and services related to your treatment. However, you and your insurer will be responsible for the remaining costs. Please contact the person listed in the contact information table to learn more about the coverage by research fund.

- If you need financial assistance to cover the cost of your treatment, please contact the staff listed in the contact information table for more information.

9. What happens if you are injured from the treatment?

Provide the following information about treatment-related injuries:

Describe any compensation and medical treatments available to the patient if injury occurs.

Provide the names and contact information of the staff whom the patient should contact if further information is needed. The available compensation and medical treatments may vary depending on the medical circumstances of the patient or the policies of the institution.

Examples:

- If this treatment results in an injury, [INSTITUTION] will provide you with medical care.

- Cost for care related to treatment-related injuries will be billed in the ordinary manner to you or your insurance company.

10. Who may see, use, or share your health information?

Provide information about the confidentiality policy of the clinic/hospital/sponsor OR include the list of your policies. Include a statement that the data from this investigational treatment will be shared with FDA and note the possibility that FDA may inspect the records related to the investigational treatment.

Examples:

- If you receive this investigational drug, certain information about your treatment may be shared with the following entities, but every effort will be made to keep your identity private:
  - The manufacturer of the drug
  - The Food and Drug Administration
  - The institutional review board
- In addition, the following people/institutions may have access to your identity and information about your use of the investigational drug:
  - Your insurance company or health benefits program
  - The clinic staff directly involved in your medical care
- If you stop treatment, information that was already collected may still be shared with FDA.
- If the result of this treatment is published, your personal identifying information will not be used.
- Although it is unlikely to happen, there is a possibility that your personal information will be disclosed accidentally.

11. What other important information do you need to know?

Provide a list of other important information not covered in the sections above.

Examples:

- During your treatment, if we learn any new information about the risks or benefits of the investigational drug Y, Dr. Z will let you know.
- You will not receive any payment as compensation to take the investigational drug Y.
- You may review our web-based, interactive educational program for patients with your disease at the following link: [insert URL link].

Whom should I contact?

Provide consolidated contact information, as appropriate. If the contact information changes at any time, provide the new contact information to the patient.

Examples:

Name **(Name of the doctor/clinical staff/board/IRB/advocate, etc.)**	**Contact information** **(Phone number, email, or address, etc., as appropriate)**	**For questions about…** **(Provide a consolidated list of issues for which a patient may have questions)**
Name of the staff	Phone: E-mail: Address:	• Treatment, including any injury from the treatment • Emergency contact information, including 24-hour contact information, if appropriate
Name of the staff/board/IRB	Phone: E-mail: Address:	• Administrative concerns (e.g., patient rights, billing)

12. Permission Signatures

Include the list of signatories who should provide consent for the treatment. Provide instructions for the assent process, if you have any specific policies.

Examples:

Your signature below provides your consent to take part in this investigational treatment.

_____

Name of patient

_____ _____

Signature of patient Date Time

_____

Name of legally authorized representative (if needed)

_____ _____

Signature of legally authorized representative (if needed) Date Time

_____

Legally authorized representative's relationship to patient (if needed)

Add any other signatures, following your institutional policy.