Participant Verbal Informed Consent Form
For Participants in the United States

Sponsor / Study Title:	**Catalyst Pharmaceuticals, Inc. / "PREGNANCY SURVEILLANCE PROGRAM OF WOMEN AND INFANTS EXPOSED TO FIRDAPSE® DURING PREGNANCY"**
Protocol Number:	**LMS-004**
Principal Investigator: (Study Doctor)	**Amy Miller, RPh, PharmD**
Telephone:	**1-855-212-5856(24-Hours)**
Address:	**UBC** **933 Canyon Road** **Morgantown, WV 26508**

INTRODUCTION

Catalyst Pharmaceuticals, Inc., the study Sponsor, has set up this voluntary observational pregnancy study to describe the pregnancy complications and outcomes of women and their infants who were exposed to at least 1 dose of Firdapse at any time during pregnancy. It is anticipated that approximately 5 pregnancies will be reported over the data collection period. Catalyst Pharmaceuticals, Inc. has contracted and is working with United BioSource (UBC) / Pregnancy Coordinating Center (PCC) to conduct this research study.

You and your baby resulting from your pregnancy are being asked to participate in this research study because you have been exposed to at least one dose of Firdapse®, or because you have Lambert-Eaton myasthenic syndrome (LEMS) and you were not exposed to Firdapse during pregnancy. This form will explain the purpose of this research study and other important information. You need to provide only your verbal consent to enroll in the study; therefore, with your permission we would like to record our consent discussion for the study's files. At the end of the discussion about joining the study, we will ask if you would like to verbally consent to enroll in the study.

RISKS

This is an observational study. There is no additional medical intervention outside of your normal standard of care that you are receiving at your doctor or other licensed medical practitioner's office. All data that is collected as part of this study is taken from the information that your doctor has documented in your medical notes during your normal doctor's visits, as well as the results of any tests that were performed during these visits.

There are no additional medical risks for you or your baby when you participate in this observational pregnancy study. While every effort will be made to safeguard your personal information, there is a small risk that your and your baby's information may be unintentionally disclosed. For this reason, absolute confidentiality cannot be guaranteed.

BENEFITS

There is no direct benefit for you or your baby for participating in this study. However, your participation in this study will help Catalyst Pharmaceuticals, Inc. to determine if there are any effects of Firdapse® on pregnant women or babies whose mothers were exposed to Firdapse® during pregnancy. The study data will be provided to regulatory agencies so that other women who become pregnant while being treated with Firdapse® can better understand the effects of Firdapse® on pregnant women and their babies.

PARTICIPATION

Your participation in this study is strictly voluntary. To participate in the study, you will be asked to do the following:

- Verbally state that you want to participate in the study (also known as verbal informed consent). You need to provide only your verbal consent to enroll in the study; therefore, with your permission we would like to record our consent discussion for the study's files. At the end of our discussion about joining the study, we will ask if would like to verbally consent to enroll in the study. We will be mailing/giving a copy of this consent to you for your files.
- Once the Pregnancy Coordinating Center (PCC) has your verbal informed consent, they will send you a Medical Information Release (MIR) form to sign, date and return. By signing and dating the MIR form, you give permission to the PCC to contact your doctor or other licensed medical practitioner and your baby's doctor or other licensed medical practitioner for medical information.
- Provide information to the PCC at the time of enrollment (at time of verbal consent) and additional information once per trimester during your pregnancy and at the following timepoints:
    - Pre-natal follow up visit at 34 weeks (Obstetric health care provider will be contacted)
    - At the estimated date of delivery, and;
    - When your baby is 3, 6, 9, and 12 months of age.

INFORMATION

During enrollment, the PCC will ask you basic questions about your health and pregnancy, as well as your contact information, including your address and phone number. The PCC will also ask you to identify two secondary contacts. The secondary contacts must be someone outside of your household who are able to contact you in case the PCC is unable to reach you.

You will be contacted by the PCC one time during each trimester of your pregnancy, on the estimated date of delivery, and when your baby is 3, 6, 9 and 12 months of age, as needed. The PCC will collect the following information:

- Any changes in the contact information you provided at enrollment
- Any changes in the status of your pregnancy
- Any changes in Firdapse® treatment, if applicable, and changes in other medications
- Any pre-natal testing at the pre-natal follow up visit
- Any changes to your baby's health status when you are contacted when your baby is 3, 6, 9 and 12 months of age

In addition, your doctor or other licensed medical practitioner who is caring for you during pregnancy will be contacted at the initial pregnancy report, around 34 weeks of your pregnancy and, again, within 2 weeks of your estimated delivery date. The PCC will also contact your baby's doctor or other licensed medical practitioner when your baby is approximately 3, 6, 9 and 12 months old to determine if there are any changes in your baby's health status.

COMPENSATION AND STUDY-RELATED EXPENSES

This pregnancy surveillance program is being sponsored by Catalyst Pharmaceuticals, Inc.; the PCC is being paid by Catalyst Pharmaceuticals, Inc., to conduct the study.

During your participation, you will not be paid for the study-required phone calls described in this informed consent form.

There are no additional costs for your participation in this study. While you are in this study, the cost of your usual medical care, procedures, medications and doctor visits, will continue to be billed to you or your insurance.

POSTING OF RESEARCH STUDY ON WEB

A final study report will be reported at the conclusion of the study and will be available on *http://www.ClinicalTrials.gov*, as guidance provided by (STROBE). This Web site will not include information that can identify you. At most, the Web site will include a summary of the final study results.

A description of this study will also be available on FDA-Pregnancy-Registries@fds.hhs.gov the Food and Drug Administration (FDA) Women's Health Research web site.

PRIVACY

There is a small risk that your and your baby's information may be inappropriately disclosed. This means absolute confidentiality cannot be guaranteed.

This study will remain open for a minimum of 10 years. Your information will remain at the PCC until approximately 2 years after the end of the study.

Information about your health collected while you are in this study (protected health information, or "PHI") will be kept in confidence and in accordance with privacy statutes and regulations (for example, the Health Information Portability and Accountability Act, or "HIPAA"). As is

customary, the Sponsor of the study, Catalyst Pharmaceuticals, Inc., may be required to provide certain safety information to the Institutional Review Board (IRB) and the FDA including personal medical information. You will need to agree to the "Authorization to Use and Disclose Protected Health Information" provided at the end of this consent form in order to authorize the use and disclosure of your PHI under HIPAA for the purposes of this study. In any presentation of the results of the study at meetings or in publications, your identity will remain anonymous and confidential.

WITHDRAWAL

Enrollment in the Firdapse Pregnancy Study is completely voluntary. You may leave the study for any reason at any time. If you decide to stop participating, the quality of your and your baby's medical care will not be affected, and you and your baby will not be penalized or lose any benefits that you and your baby may be entitled to. If you decide to leave the study before your participation has ended, Catalyst Pharmaceuticals, Inc., will still use the information collected before your withdrawal. The request for withdrawal from the study must be made to the PCC by you or your health care provider. The study investigator or the Sponsor can stop the study at any time without your consent.

The Investigator or the Sponsor can stop your participation at any time without your consent for the following reasons:

- If you fail to follow directions for participating in the study;
- If it is discovered that you do not meet the study requirements;
- If the study is canceled; or
- For administrative reasons.

NEW FINDINGS

Any new important information that is discovered during the study and which may influence your willingness to continue participation in the study will be provided to you.

ALTERNATIVES TO PARTICIPATION

This pregnancy follow-up is for research purposes only. The only alternative is to not participate in this study.

WHOM TO CONTACT ABOUT THIS STUDY

During the study, if you have questions, concerns or complaints about the study, please contact the Investigator at the telephone number listed on the first page of this consent document.

An IRB is an independent committee established to help protect the rights of research participants. If you have any questions about your rights as a research participant, and/or concerns or complaints regarding this research study, contact**:**

- By mail:
  Study Participant Adviser
  Advarra IRB
- 6100 Merriweather Dr., Suite 600
  Columbia, MD 21044
- or call **toll free**: 877-992-4724
- or by **email**: adviser@advarra.com

Please reference the following number when contacting the Study Participant Adviser: Pro00045362.

A copy of this Participant Informed Consent Form will be provided/mailed to you for your records. The PCC will sign and date this form if you choose to participate.

We will also include the MIR form that you will need to sign, date and return in the self-addressed and pre-stamped envelope.

PCC Associate reviewing Participant Informed Consent Form:

Printed name/Signature of PCC Associate	Date Signed

Printed name of Study Participant

AUTHORIZATION TO USE AND DISCLOSE PROTECTED HEALTH INFORMATION

If you decide for you and your newborn child to be in this study, the Study Investigator and research team will use and share health data about you and your newborn child to conduct the study. Health data may include:

- Name.
- Address.
- Phone number.
- Date of birth.
- Medical history.
- Information from your doctor visits, including test results.

Health data may come from your and your newborn child's study records or from existing records kept by your and your newborn child's doctor or other health care workers.

For this study, the research team may share health data about you and your newborn child with authorized users. Authorized users may include:

- Representatives of Catalyst Pharmaceuticals, Inc.
- United BioSource (UBC) / Pregnancy Coordinating Center (PCC)
- Representatives of Advarra IRB (an Institutional Review Board that reviews this study).
- The Food and Drug Administration (FDA) and other U.S. federal and state agencies.
- Government agencies to whom certain diseases (like HIV, hepatitis, and STDs) must be reported.
- Governmental agencies of other countries (for example, Health Canada).
- Outside individuals and companies, such as laboratories and data storage companies, that work with the researchers and Sponsor and need to access your information to conduct this study.
- Other research doctors and medical centers participating in this research, if applicable.
- A data safety monitoring board which oversees this research, if applicable.

Your and your newborn child's health data will be used to conduct and oversee the research, including for instance:

- The frequency of pregnancy complications in women who were exposed to at least 1 dose of Firdapse® during pregnancy and fetal and infant outcomes in infants through 1 year of age.

- The frequency of adverse events affecting lactation in women with LEMS and infants through 1 year of age who have not been exposed to Firdapse® during pregnancy.

Once you and your newborn child's health data has been shared with authorized users, it may no longer be protected by federal privacy law and could possibly be used or disclosed in ways other than those listed here.

Your permission to use and share health data about you and your newborn child will end in 50 years unless you revoke it (take it back) sooner.

You may revoke (take back) your permission to use and share health data about you and your newborn child at any time by writing to the Study Investigator at the address listed on the first page of this form. If you do this, you and your newborn child will not be able to stay in this study. No new health data that identifies you or your newborn child will be gathered after your written request is received. However, health data about you and your newborn child that has already been gathered may still be used and given to others as described in this form.

Your right to access your and your newborn child's health data in the study records will be suspended during the study to keep from changing the study results. When the study is over, you can access your/your newborn child's study health data.

If you decide not to provide your authorization, you and your newborn child will not be able to take part in the study.

I voluntarily agree to allow study staff to collect, use and share my and my newborn child's health data as specified in this form. I am not giving up any of my or my newborn child's legal rights by providing my authorization.

Firdapse® PCC Associate reviewing Authorization to Use and Disclose Protected Health Information:

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Printed name/Signature of PCC Associate Date Signed

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Printed name of Study Participant