[Letterhead of participating CINRG site to be inserted]

GENERAL INSTRUCTIONS FOR SITES TO COMPLETE THE LOCAL CONSENTS:

THIS IS A TEMPLATE AND SHOULD BE MODIFIED TO CONFORM TO YOUR LOCAL IRB/ETHICS BOARD'S REQUIREMENTS.

- WHEN FILLING IN THE BLANKS, PLEASE MAKE SURE THE INSTRUCTIONS IN GREY BACKGROUND ARE THEN DELETED.
- IF YOUR LOCAL IRB/ETHICS BOARD REQUIRES PARTICIPANT/PARENT INITIAL ON EACH PAGE PLEASE ADD THE NECESSARY LINES, ETC.
- PLEASE CONTACT THE PROJECT MANAGEMENT TEAM AT THE COORDINATING CENTER IF YOU HAVE ANY QUESTIONS AS YOU ARE DRAFTING YOUR SITE'S CONSENT FORM.
- AS A REMINDER, YOUR CONSENT FORM MUST BE REVIEWED BY THE COORDINATING CENTER BEFORE SUBMISSION TO YOUR IRB/ETHICS BOARD.

CONSENT TO PARTICIPATE IN A CLINICAL RESEARCH STUDY AND AUTHORIZATION TO USE PROTECTED HEALTH INFORMATION

TITLE OF STUDY: **A 24-month Phase II Open-Label, Multicenter Long-Term Extension Study to Assess the Long-term Safety and Efficacy of Vamorolone in Boys with Duchenne Muscular Dystrophy (DMD)**

Protocol Number: VBP15-LTE

STUDY DOCTOR: [Name of participating site PI to be inserted]

SPONSOR: ReveraGen BioPharma, Inc.

STUDY CHAIR: The Study Chair is Dr. Paula Clemens from the University of Pittsburgh.

INTRODUCTION: We would like to invite your son or legal ward (referred to as "son" in the remainder of the document) to be part of a multicenter research study at [Name of participating CINRG site to be inserted]. [(Name of participating CINRG Site to be inserted] is a member of the academic research group: Cooperative International Neuromuscular Research Group (CINRG). The study's Coordinating Centers support various aspects of the trial procedures (for example, data management, safety procedures). Dr. Paula Clemens at the University of Pittsburgh in Pittsburgh, PA, USA is the study chair.

Before you decide if you would like your son to participate, we want you to know why we are doing the study. We also want you to know about any risks (anything unexpected that might happen) and what you and your son will be expected to do in the study.

This form gives you information about the study. Your doctor will talk to you and your son about the study and answer any questions you have. We encourage you to discuss this study with your family and anyone else you trust before making your decision. We will ask you to sign this form to show that you understand the study. We will give you a copy of this form to keep.

It is important that you know:

- Your son does not have to join the study;
- You may change your mind and stop your son being in the study any time you want. However, we will ask for your son to be seen by the study team so that the study can be stopped safely;
- If we make any important change to the study we will tell you about it and make sure you still want your son to be in the study.

GENERAL STUDY INFORMATION

1. The main purposes of this study are to see if it is safe to use an investigational drug called vamorolone long-term in children with Duchenne muscular dystrophy (DMD) and to study if boys with DMD who take vamorolone have improved muscle function compared to boys with DMD in other studies who do not take any type of steroid. As you know from your son's participation in earlier studies of vamorolone, vamorolone is a potential new type of steroid drug that may have fewer side effects compared to traditional steroids like prednisone and deflazacort. Steroids are currently the only known class of medication that has been shown to prolong walking ability in DMD.

2. The investigational drug to be used in this study is vamorolone. It will be administered as a flavored liquid suspension. Investigational means that vamorolone has not yet been approved by [insert regulatory body relevant to participating CINRG site].

3. Boys who participate in the study will take vamorolone daily for 24 months. For at least the first month of each boy's participation in this study, he will take the same dose of vamorolone that he was taking at the VBP15-003 Week 24 visit (0.25 mg/kg/day, 0.75 mg/kg/day, 2.0 mg/kg/day, or 6.0 mg/kg/day). After one month, boys may be allowed to start taking the next higher dose. For example, boys starting this study on 0.25 mg/kg/day may be allowed to start taking 0.75 mg/kg/day after 1 month. For boys starting on 6.0 mg/kg/day in this study, they will remain on this, or a lower dose throughout the study.

4. There may be changes to this plan based on any problems experienced by the participating boy, or other boys in the study. This study is open label, which means that both you and the Study Doctor will know the treatment your son is receiving.

5. We believe that your son may be eligible to participate in this research study because he was able to take vamorolone in the VBP15-002 and VBP15-003 studies; he completed the last study visit for VBP15-003 study and appears to meet other requirements to join the study.

6. We expect [insert estimated number of participants that plan to be enrolled at local site] boys with DMD to enroll in the study at [Name of participating CINRG site to be inserted]. Approximately 48 boys are expected to participate at approximately 15 CINRG centers across multiple countries. All boys who were able to take vamorolone in the VBP15-002 and VBP15-003 studies, successfully completed their last visit for the VBP15-003 study, and remain eligible for this study will be invited to participate in this extension study.

7. A description of this clinical trial is available on http://www.ClinicalTrials.gov, as required by U.S. Law. This website will not include information that can identify you or your son. At most, the website will include a summary of the results. You can search this website at any time.

RESEARCH PROCEDURES

Your son will be in the study approximately 24 months if he is found to be eligible. Each visit for the study is described in this form. Appendix I Study Schedule on the last page of this form displays the research visits and what procedures are to occur at each visit. All procedures are done for research purposes and are outside of the normal care that your son would receive if he did not participate in this study.

Pre-Treatment Period: 1 In-Person Visit

BASELINE VISIT (Day -1: Within 24 Hours of Starting Vamorolone in this study)
The baseline visit takes place during the 24 hours prior to starting vamorolone for this long-term extension study. It can be combined with the VBP15-003 Week 24 visit or take place up to 8 weeks after the VBP15-003 Dose-Tapering Visit. If the Day -1 visit takes place on the same day as or within 28 days after the VBP15-003 Week 24 visit, not all of the procedures below will be repeated. At the Baseline Visit the following procedures will occur:

- Consent: A member of the study team will review this consent document with you and your son, will answer any of your questions, and obtain your signature.
- Medical and Medication History: A member of the study team will ask you about any changes to your son's medical history or medications, including over-the-counter medications, since he completed the VBP15-003 study.
- Physical exam: A physical exam will be performed by a study doctor.
- Adverse Events Review: At the end of the visit a member of the study team will ask you about any adverse events (illnesses, injuries, or other undesirable experiences) that your son has experienced since you signed the consent and any updates to adverse events experienced in the VBP15-002 or VBP15-003 study.
- Vital signs: A member of the study team will collect your son's blood pressure, oral temperature, breathing rate, heart rate, height, and weight.
- Urine Collection: The study team will collect a urine sample to help determine if there are any health concerns for your son to enter the study or to continue to receive vamorolone.
- Blood collection: A trained member of the study team will collect blood from a vein in your son's arm. The blood will be analyzed to help determine if there are any health concerns for your son to enter the study and your son's baseline or starting level of certain markers in his blood. These markers are called pharmacodynamic biomarkers as they may change while your son is taking vamorolone. These biomarkers will be looked at in your son's blood

throughout the study to help determine if vamorolone may be helpful and to assess any side effects that it may be causing. Your son's blood will be analyzed at commercial laboratories hired by ReveraGen for this purpose. The information collected from these analyses, together with other information collected about your son from the study, will be studied by ReveraGen and its representatives.

- Electrocardiogram (ECG): This is a test that gives a measure of the electrical activity of the heart. Your son will be asked to lie flat on a table and several small electrode pads (like stickers) will be placed on his body.
- Muscle strength and functional tests: A trained physical therapist will ask your son to complete a number of assessments to study his muscle strength and ability to perform several tasks. These are the same assessments he completed in the VBP15-002 and VBP15-003 studies. To measure muscle strength, your son will sit/lie on an examining table and will be asked to pull/push a strap. The functional assessments will include tasks such as stepping on to a step, jumping, and hopping on one leg. During the timed tests, the trained physical therapist will collect the time that it takes for your son to complete different tasks (walk or run a 10 meter (approximately 33 feet) course, stand from a lying position, and climb 4 stairs). He will also be asked to walk for 6 minutes and the distance he walks during those 6 minutes will be recorded.
- Quality of life questionnaire: You will be asked to complete a questionnaire regarding your son's qualify of life. This questionnaire is called the Pediatric Outcomes Data Collection Instrument.

You will receive enough vamorolone for your son to take the study drug at home every day until the Month 3 Visit. You will receive instructions on how to give vamorolone to your son. Study diaries and directions to complete the study diaries will be given. The study diary will be used to record any illnesses, injuries, or other undesirable experiences that your son experiences and any medication changes.

This visit will take approximately 1.5-4 hours.

Treatment Period: 9 In-Person Visits

During the 2-year Study Treatment Period, your son will be taking vamorolone once every morning. Your son will start the study taking the same dose of vamorolone that he was taking at the VBP15-003 Week 24 visit. His dose may increase after the Month 1 visit (except participants in the 6.0mg/kg/day group), or may decrease as needed for safety reasons. If your son's dose is decreased from 6.0mg/kg/day to 2.0mg/kg/day, and after at least 1 month it is determined that vamorolone isn't working as well, his Study Doctor may consider increasing the dose to 4.0 mg/kg/day. Your study team will direct you as to what dose your son should be taking.

Vamorolone will be taken at home every day during this period EXCEPT on the days of the Month 9 and Month 21 visits, when your son will fast at least 6 hours, arrive at the study site in the morning fasted for a pre-dose blood draw and then eat breakfast and take vamorolone during the study visit at [Name of participating CINRG site to be inserted] following the directions of the study team. Your son must take vamorolone with 8 ounces [replace with equivalent volume in appropriate units if necessary] of whole fat milk or a different food or drink with a

similar amount of fat (approximately 8 grams) every morning. A three-month supply of study medication will be given to you at each visit (except the Month 1 visit and the Month 24 visit). At each visit during which you receive study medication, you will return the unused portion of the medication supply given to you at the previous visit.

During this period you will be asked to record any illnesses, injuries, or undesirable experiences (adverse events) that your son experiences and any changes in medications. The study diaries will be provided by the study team and collected at each in-person visit except the Month 1 Visit when the study team will review your diary and return it to you. Serious illnesses, injuries, or other undesirable experiences that occur during this time period should be reported immediately to the study team at [Name of participating CINRG site to be inserted] by contacting [Name of contact at participating CINRG site] at [Contact information]. You should try to avoid making any changes to your son's medications including over-the-counter medications during this time period if possible. If there are any changes to your son's medications including non-prescription, over-the-counter medications (starting medications, stopping medications, changing medication doses), these should be tracked in the study diaries and reported to the study team at [Name of participating CINRG site to be inserted]. Medicines belonging to the group called mineralocorticoid receptor agents, idebenone, steroids or other immune suppressing medicines taken by mouth, live vaccines, and any other investigational medications should not be taken during this period or throughout the duration of the study.

Study Day 1 (at home):
On Day 1, your son will take vamorolone for the first time in this long-term study. He will take vamorolone at home. A visit to [Name of participating CINRG site to be inserted] is not required on this day.

Months 1, 3, 6, 9, 12, 15, 18, and 21 visits:
You and your son will return to [Name of participating CINRG site to be inserted] 1 month after starting vamorolone in this study, 2 months later, and then every 3 months until the Month 24 visit. Vamorolone will be taken at [Name of participating CINRG site to be inserted] on the days of the Month 9 and Month 21 visit and your son will have to fast at least 6 hours for these visits. A three-month supply of vamorolone will be given to you at the Baseline Day -1 and each subsequent visit (except the Month 1 visit). At each subsequent visit, you will return the unused portion of the vamorolone supply given to you at the previous visit.

The additional procedures that will be completed at each visit are outlined in Appendix I Study Schedule on the last page of this document and are described in the baseline visit above. The length of these visits will vary but will be between 30 minutes and 3 hours.

Month 24 Visit
This is your son's final visit for the study if he will continue taking vamorolone after the study completion and is therefore not participating in the dose-tapering period. If your son will not continue taking vamorolone after study completion and therefore is required to participate in vamorolone dose-tapering, you will receive additional vamorolone at this visit and instructions for how to slowly and safely reduce the amount of vamorolone your son is taking over 1-4 weeks.

At this visit the following procedures will occur:

- Blood collection: In addition to the blood tests described in the baseline visit above, your son's blood will be studied for certain genes that are known to affect DMD severity or response to other steroid treatments to see if these gene effects are also associated with how well vamorolone treatment works in DMD.
- Spine x-ray: A technician will take x-rays of your son's back to look for spine fractures (called compression fractures), which are fractures that might occur when the bones start to get weak. These compression fractures in the spine are often silent (without pain), which is why an x-ray is needed to diagnose them. During the spine x-ray, your son will have to stay still for a few seconds while the x-ray is being taken.
- Hand x-ray: A technician will take an x-ray of your son's hand to determine his bone age. Bone age testing is done to help to determine how quickly or slowly a child's skeleton is growing and may be used to diagnose delays in physical growth. During the hand x-ray, your son will be asked to hold his hand still with his fingers spread while the x-ray is being taken.

Additional procedures that will be completed at this visit are outlined in Appendix I Study Schedule on the last page of this document and are described in the baseline visit above. This visit will take approximately 5 hours.

You should discuss options for your son's medical care following completion of the study with your son's Study Doctor and your son's other doctors.

Post-Treatment Period: 0 or 1 In-Person Visit

If your son will not continue taking vamorolone after completion of the study, his vamorolone dose will be reduced slowly during this period. Depending on your son's starting dose of vamorolone, this will take between 1 and 4 weeks. The drug taper is done for your son's safety. Your son's Study Doctor will provide instructions for dose tapering. If your son will take another steroid medication after completion of the study, he will start taking that medication on the day the dose-tapering begins and continue on this dose throughout the Post-treatment Period, as directed by your Study Doctor. Your son's medical doctor will manage your son's treatment with this medication after his participation in the study is complete.

Month 24-25 Visit

If your son completed the dose tapering, you and your son will return to [Name of participating CINRG site to be inserted] approximately one week after your son takes his last dose of vamorolone (approximately 2-5 weeks after dose-tapering begins). At this visit, your son will have vital signs and a blood draw and you will return the unused portion of the vamorolone supply given to you at the previous visit, as well as the completed study diaries. This visit will take approximately 1 hour.

WHAT ARE THE POTENTIAL RISKS OR DISCOMFORTS?

Your son might experience some side effects or discomfort while participating in this research. The laboratory tests, physician exams, and adverse event review are designed to help monitor and reduce the chance of developing side effects. If side effects develop, please contact [Name of contact at participating CINRG site] at [Contact information], who will work to reduce

or eliminate those side effects. The side effects that your son could experience are listed below. There could be side effects that are unknown.

1. Risks of Taking the Study Drug

   ADRENAL SUPPRESSION
   After taking vamorolone for 24 weeks in the VBP15-003 study, adrenal suppression was seen at the 2 higher doses of vamorolone: Five of 12 boys (42%) taking 2.0 mg/kg/day, and 8 of 9 boys (89%) taking 6.0 mg/kg/day experienced adrenal suppression. This was noted in blood tests only (decreased cortisol levels) and did not result in any symptoms.

   Adrenal suppression is caused by decreased functioning of the adrenal glands. The adrenal glands produce hormones in the body. Often children with adrenal suppression do not have any symptoms or have non-specific symptoms. Signs and symptoms of adrenal insufficiency may include:

   - Fatigue/tiredness
   - Nausea/vomiting
   - Abdominal/stomach pain
   - Muscle pain
   - Muscle weakness
   - Dizziness
   - Trouble thinking clearly
   - Poor growth
   - Weight loss
   - Behavior changes

   Adrenal suppression can also be seen in individuals who are taking or recently stopped taking traditional steroids (prednisone, deflazacort).

   Some of these signs and symptoms are also seen in people with DMD without any adrenal problems.

   Children with adrenal suppression are at risk of becoming very ill. This is rare but when this happens, it is called adrenal crisis. Adrenal crisis can be associated with low blood pressure and/or low blood sugar and typically occurs when a child with unrecognized adrenal insufficiency has a physical stress such as an illness, surgery, or injury. No healthy adult volunteers taking vamorolone in the Phase I clinical trials experienced adrenal crisis. No subject participating in the VBP15-002 and VBP15-003 studies experienced adrenal crisis. Adrenal crisis can be prevented by providing steroid replacement in “stress doses” during the acute stress.

   If your son has any of the symptoms above, his Study Doctor should be notified. A referral to an endocrinologist with consideration of testing of adrenal function and provision of replacement steroids may be made. Also, if your son requires a surgical procedure, has a serious injury or develops an illness during participation in this study, you should inform

your Study Doctor immediately so that they can advise you on minimizing the risk of adverse events due to possible adrenal insufficiency.

ELEVATED LIVER ENZYMES

In a previous study of vamorolone in healthy adult volunteers, one adult in the highest dose group (20 mg/kg/day) experienced transient elevated liver enzymes, which could reflect potential liver damage. Thus, elevated liver enzymes and liver damage are also a possible risk associated with vamorolone. The most common blood tests to check for liver problems are AST and ALT enzymes. In patients with DMD, however, these enzymes are already elevated due to muscle damage and therefore these enzymes cannot be used to assess liver damage problems in DMD patients. Therefore, potential liver damage problems were monitored in the VBP15-002 and VBP15-003 studies of vamorolone in boys with DMD by testing for other liver-preferential enzymes that are not elevated in DMD. These results were re-assuring; however, more information is needed to better assess risk of elevated liver enzymes in DMD due to vamorolone. In the current study, liver-preferential enzymes are being monitored.

WEIGHT GAIN

From the beginning of VBP15-002 study through the end of VBP15-003 study, boys on the highest dose of vamorolone (6.0mg/kg/day) had greater increases in BMI (body mass index) than boys on the lower doses. This increase in BMI was similar to the BMI increase noted in boys taking prednisone for a similar amount of time.

Additional information regarding any risks associated with the study drug for boys with DMD from the VBP15-002 and VBP15-003 studies may be available at the time of your son's enrollment and should be discussed with your son's Study Doctor. Other risks associated with taking the study drug in boys with DMD are unknown at this time including potential effects on long term development and reproductive development.

2. Risks of Blood Draws:
   Blood draw may cause soreness or bruising at the site of the needle insertion. This is a common risk. Rarely, lightheadedness, fainting, or a more serious injury, such as hematoma (bleeding under the skin) may develop. To reduce the discomfort of the blood draw, a local numbing cream may be applied to the area. The side effects that may be associated with numbing cream include lack of sensation to the area where it is applied with an increased chance of harm to the area because of lack of sensation.

   At most visits, between 19 and 22 milliliters (approximately 4 teaspoons) of blood will be collected. At the Month 9 and Month 21 visits, 5 milliliters (approximately one teaspoon) will be collected.

3. Risk of Electrocardiography:
   Rarely, this test may cause irritation to the skin under the electrodes.

4. Risks of Muscle Strength, Functional, and Timed Tests:
   It is possible that this testing could make your son more tired than after a regular (non-

research) doctor's visit or that he may have muscle soreness. These are common risks. There are also uncommon risks of falling or shortness of breath.

5. Risk of X-Rays
   During the spine x-ray and the hand x-ray, your son will be exposed to a small amount of radiation. The extra radiation to which your son will be exposed because your son is participating in this study is less than your son would be exposed in a normal year. This is considered an acceptable amount of radiation for patients with a high risk of fractures like in DMD.

6. Risk of Loss of Confidentiality:
   The confidentiality of all study-related records will be maintained in accordance with state and federal laws. All paper records containing identifying information will be kept in locked files accessible only to the study team and unlocked only while a study staff member is physically present. Results of the study procedures listed above (without any identifying personal data) will be entered into a secure study database for analysis. Your son will only be identified by a study ID number to protect his confidentiality. However, there is a risk of loss of confidentiality which is rare.

WHAT ARE THE POTENTIAL BENEFITS?

Your son may experience a delay in muscle function decline compared to boys with DMD who are not treated with steroids and/or he may experience fewer side effects than boys with DMD who are treated with other types of steroids.

Overall, an improvement was seen in the VBP15-003 study in the time that it took boys taking 2.0 mg/kg/day and 6.0 mg/kg/day to complete some of the functional assessments completed as part of the study, such as the Time to Stand Test, Time to Run/Walk 10 Meters, and 6-Minute Walk Test. The improvement seen varied between boys but was approximately similar to the improvement seen by taking prednisone for a similar amount of time and was significantly better than changes in these functional assessments in boys who did not take vamorolone or any other steroids. Based on blood tests, there is also the possibility of more stable glucose and bone metabolism with vamorolone compared to prednisone.

Based on this information, it is possible that your son will gain some improvement from vamorolone by participating in this study as compared to not taking any steroids or as compared to taking prednisone. However, it is unknown if taking vamorolone for a period of longer than 24 weeks will improve strength and/or function further. Therefore, it is still possible that he will gain no additional personal benefit from participating in this study.

In addition, others may possibly benefit from information that the doctors gain while treating your son.

IS MY PARTICIPATION VOLUNTARY?

It is your choice to have your son take part in this study. You can stop his participation at any time. There is no penalty or loss of benefit to which you and your son are otherwise entitled if you decline to have your son participate or withdraw him from the study. The care your son

receives at [Name of participating CINRG site to be inserted] will not be affected in any way by whether he takes part in this study.

Your child's doctor may be involved as an investigator in this study. As both doctor and a research investigator, s/he is interested both in your child's medical care and the conduct of this research study. Before agreeing to participate in this screening, or at any time during your study participation, you may discuss your child's care with another doctor who is not associated with this study. You are not under any obligation for your child to participate in any research study offered by your doctor.

Any data and blood samples collected up to the point of withdrawal may be used for study purposes. Your decision to withdraw your son's agreement for the use of your son's private health information for this research study will have no effect on you or your son's current or future medical care at [Name of participating site to be inserted].

One of the investigators may withdraw your son from the study for various reasons including if: your son cannot or does not take vamorolone, you or your son do not comply with study procedures, safety blood test results indicate that continuing in the study could be harmful, we are unable to obtain blood samples from your son for safety monitoring, your son experiences side effects that are not tolerable, or if the study is closed.

If your son withdraws from the study before its completion, we will ask your son to return to [Name of participating site to be inserted] for two final study visits as soon as possible after he withdraws. If you wish to withdraw your informed consent, a written letter should be sent to [Insert PI Name] at [Insert Address] indicating that you are withdrawing consent and the date of withdrawal. In this case, no further study procedures will be performed and no additional data will be collected.

WHAT ARE MY ALTERNATIVES TO PARTICIPATION?

If you choose not to have your son participate in this study, standard medical care will be offered including initiation of traditional steroid therapy. Steroid therapy has been shown to prolong the ability to walk in boys with DMD. Steroid therapy has been associated with side effects such as weight gain, loss of bone density, adrenal suppression, and behavior changes. Other research studies may also be available to your son. You should further discuss these options with your son's Study Doctor or your son's other doctors.

IF I HAVE QUESTIONS WHO DO I CALL?

We want you to ask questions about any part of this study or consent form either now or at any time in the future. If you have any questions about this study, call the Study Doctor, [Insert PI Name], at [Insert Area Code and Number]. If you have any questions or concerns about your son's rights in this research study at any time, please call [Name and telephone number of participating CINRG site ethics or patient advocate to be inserted].

HOW WILL MY CONFIDENTIALITY BE MAINTAINED?

We will keep the records of this study confidential. Study records will be kept for at least as long as required by local law, institutional requirements, or relevant regulatory authorities in all regions participating in this study, whichever is longer. [Insert applicable study retention policies of participating CINRG site. For U.S. sites include: FDA regulations require that study records are kept for 2 years following the date that the FDA approves the study drug for use in DMD; or, if the sponsor does not submit an application for approval of the study drug for DMD or the FDA does not approve the study drug for DMD, the study records will be kept for at least 2 years after this study is discontinued and FDA is notified].

The people working on the study at [Name of site to be inserted] will know your son's name and they will keep this information in case we need to find you later to let you know of any new information that may affect your son's health. The following entities may review the study records and medical records (including your son's identifying information in rare cases) to make sure that the study is carried out correctly and that we are following the law and protecting the children in the study: US Food and Drug Administration, the study's Coordinating Centers, the study sponsor ReveraGen BioPharma and its representatives, the National Institutes of Health (NIH), and the Institutional Review Board or ethics board overseeing the study activities at [Name of site to be inserted]. Data obtained from this study may be shared with other investigators interested in DMD. However, nothing shared will contain information that can identify your son. Your son's medical record is confidential, but just like any medical record, there are some exceptions under state and federal law.

[U.S. site only: Insert relevant HIPAA section for local site. Other sites: insert applicable health information protection regulations.]

WILL I GET PAID FOR BEING IN THE STUDY?

You or your son will not be paid for his participation in the study. We will provide you and your son with reimbursements for any travel costs related to your visits to [Name of participating site to be inserted] for any study visits. The experimental drug, vamorolone, will be provided free of charge. You will also not be charged for additional tests and procedures that are performed only because you are participating in this research. To make it easier to carry investigational supplies home you may receive a tote bag worth approximately $1.50 US that is labeled with the sponsor's name. You will be responsible for standard of care procedures that your doctor would normally recommend. You will still have to pay for any medical care that is not part of the study.

MEDICAL CARE FOR RESEARCH RELATED INJURY

[Please enter your site-approved research related injury language consistent with the sponsor study contract]

CONSENT FOR BIOLOGICAL SAMPLE RETENTION AND USE

We are asking for your permission to store and use the biological samples (blood and urine) collected as part of this study for future research. If you give this permission, your son's samples will be linked to a study ID number. Only the study staff at [Insert contact at

participating CINRG site] have the link between the study ID number and your son's identifying information. You will not be informed of the results of any future research that uses your son's biological samples.

I DO agree for the biological samples collected from my son as part of this study to be stored indefinitely and used by ReveraGen for future research.

___ YES ___ NO

ADDITIONAL ELEMENTS

If any information is learned that might affect your son's willingness to continue participation in this research, you will be informed.

****

VOLUNTARY CONSENT:

The above information has been explained to me and all of my current questions have been answered. I understand that I am encouraged to ask questions about any aspect of this screening during the course of this study, and that such future questions will be answered by a qualified individual or by the investigator(s) listed on the first page of this consent document at the telephone number(s) given. I understand that I may always request that my questions, concerns or complaints be addressed by a listed investigator.

I understand that I may contact the Human Subjects Protection Advocate at [Insert contact at participating CINRG site] to discuss problems, concerns, and questions; obtain information; offer input; or discuss situations that have occurred during my child's participation.

I understand that I am permitted access to information (including information resulting from my child's participation in this research study) contained within my child's medical records, but that I am unable to access data related to the study until the end of the research study. By signing this form, I consent for my child to participate in this research study and provide my authorization to share my child's medical records with the research team.

_____

Subject's (Child's) Name (Print)

I understand that, as a minor (age less than 18 years), the above-named child is not permitted to participate in this screening without my consent. Therefore, by signing this form, I give my consent for his participation. I know what may happen, both the possible good and bad (benefits and risks). I choose to have my child in this screening. I know I can stop my child from being in the screening at any time and my child will still get the usual medical care. I do not give up any of my child's legal rights by signing this informed consent form. A copy of this consent form will be given to me.

_____ _____

Parent or Guardian's Name (Print) Relationship to Subject (Child)

_____ _____

Parent or Guardian's Signature Date

For children who are developmentally able to sign name: This research screening has been explained to me and I agree to participate.

_____

Printed name of child-subject:

_____

Signature of child-subject:

I believe that my child understands what this research screening involves and that he/she has given assent for his/her participation.

Signature of parent/guardian: _____

VERIFICATION OF EXPLANATION:

I certify that I have carefully explained the purpose and nature of this research screening to the above-named child in age appropriate language. He/she has had an opportunity to discuss it with me in detail. I have answered all his/her questions and he/she has provided affirmative agreement (i.e. assent) to participate in this screening.

_____ _____ _____

Investigator's Signature Printed Name of Investigator Date

CERTIFICATION OF INFORMED CONSENT:

I certify that I have explained the nature and purpose of this screening to the above-named individual(s), and I have discussed the potential benefits and possible risks of study participation. Any questions the individual(s) have about this study have been answered, and we will always be available to address future questions as they arise. I further certify that no research component of this protocol was begun until after this consent form was signed.

_____ _____

Printed Name of Person Obtaining Consent Role in Research Study

_____ _____

Signature of Person Obtaining Consent Date

APPENDIX I STUDY SCHEDULE

	Base-line	Research Procedures										Dose-Taper
Visit/Contact	Day -1[a]	Day 1 (at home)	Month 1	Month 3	Month 6	Month 9	Month 12	Month 15	Month 18	Month 21	Month 24	Month 24-25[b]
Informed Consent	X											
Medical and Medication History	X											
Physical Examination	X						X				X	
Adverse Events Collection	X	→	→	→	→	→	→	→	→	→	→	→
Medication Changes Collection		X	→	→	→	→	→	→	→	→	→	→
Vital Signs	X		X	X	X	X	X	X	X	X	X	X
Urine Collection	X				X		X		X		X	
Blood Draw	X				X	X[h]	X		X	X[h]	X	X
Electrocardiogram (ECG)	X						X				X	
Muscle Strength, Functional, and Timed Tests	X						X				X	
Vamorolone Dosing[c]		X	→	→	→	X	→	→	→	X	→	
Vamorolone Tapering												X
Receive Study Diaries and Medication	X			X	X	X	X	X	X	X	X[d]	
Return Study Diaries and Medication			X[e]	X	X	X	X	X	X	X	X	X
Quality of Life Questionnaire	X						X				X	
Spine x-ray											X	
Hand x-ray											X	
Discharge from Study											X[f]	X[g]
Approximate Duration of Visit (hours)	1.5-4	5 min	1	1	1.5	1.5	4	1	1.5	1.5	5	1

[a] If the Baseline Visit (Day -1) occurs within 28 days after the VBP15-003 Week 24 Visit, some assessments performed at the VBP15-003 Week 24 visit will not be repeated.

[b] All participants EXCEPT those who elect to continue vamorolone therapy in a further extension study must continue in the Dose-tapering Period and have their vamorolone dose tapered at weekly intervals over a 1-4 week period prior to discharge from this

study. Subjects participating in the Dose-tapering Period will have one study site visit during this period, 1 week after the end of dose tapering.

[c] Vamorolone will be taken at home every day during the Treatment Period except on days of the Month 9 and Month 21 visits. Vamorolone will be taken at [Name of participating CINRG site to be inserted] on those days. A supply of vamorolone will be given to you at the Day -1, Month 3, and each subsequent visit (except last visit). You will return the unused portion of vamorolone given to you at each visit at the subsequent visit.

[d] Only for participants who will participate in the Dose-tapering Period.

[e] Diary is reviewed and returned to participant at Month 1 Visit.

[f] Participants who elect to continue to take vamorolone in a subsequent extension study may be discharged from the study following completion of all final Month 24 procedures.

[g] Participants who will not continue to take vamorolone will be discharged from the study following completion of the final Month 24-25 Dose-tapering Visit.

[h] Participants must be fasting (no food or liquids except water) for at least 6 hours before blood draw