Summarized Information

Summarized Information_English

Product Name	Vamorolone
Active substance	17α,21-dihydroxy-16α-methyl-pregna-1,4,9(11)-triene-3,20-dione
Indication and conditions of use	The treatment of boys with Duchenne muscular dystrophy who are completing the ReveraGen VBP15-004 trial, under the conditions that they are deriving clinical benefit from the therapy according to their physician and are not experiencing adverse events related to the treatment that precludes safe use in that patient. Medical testing and monitoring should be performed by the physician as he/she would usually conduct for a patient with DMD who is being treated with conventional corticosteroids. A card should be carried by the patient/family indicating the need for stress dose steroids in the event of serious illness or surgery. Vamorolone may be prescribed at a dose of 2, 4, or 6 mg/kg/day delivered once daily in the morning. Vamorolone will be administered by mouth using a volumetric syringe. Following administration, the syringe will be filled once with water and the water will be administered by mouth using the volumetric syringe. The patient should then drink approximately 50 mL (approximately 2 ounces) of water to ensure the full dose has been ingested.
Conditions, delays and further rules for participation of patients	**Conditions for participation:** •The Patient's parent or legal guardian has provided written informed consent under applicable Data Privacy Regulations such as GDPR. • The Patient has provided written assent, when appropriate. •Patient has previously completed VBP15-004 up to and including the Week 48 assessments. •Patient and parent/guardian are willing and able to comply with recommended vamorolone administration plan, and standard of care follow-up and monitoring as recommended by their Treating Physician. •The patient is not currently eligible for a currently enrolling clinical trial with vamorolone and/or another clinical trial enrolling patients with Duchenne muscular dystrophy. •The patient cannot be satisfactorily treated with the approved and commercially available alternative treatments, in accordance with clinical guidelines, because of safety concerns associated with corticosteroids (e.g. growth stunting, bone fragility, Cushingoid appearance, mood changes). **Conditions excluding participation:** •Patient has or had a serious or severe adverse event in study VBP15-004 or during compassionate use that, in the opinion of the Treating Physician, was probably or definitely related to vamorolone use and precludes safe use of vamorolone for the patient.

	•Patient and/or parent/guardian are unable and/or unwilling to comply with regular medical care and follow-up as recommended by their Treating Physician. A treatment request will be sent by the treating physician to the responsible physician at least 4 weeks before the Week 48 visit of the VBP15-004 trial if possible, to avoid the need for tapering vamorolone at the end of the trial. Within 3 weeks, the responsible physician should evaluate the request. Once the responsible physician has notified the Sponsor of the approval, in most cases vamorolone may be delivered to Site Pharmacy within 7 working days.
Duration of the program	Vamorolone will be provided by Santhera on an individual patient basis following the criteria stated in this program from the authorization/set-up of the Compassionate Use Program in Belgium until the product will be commercially available in Belgium in Duchenne muscular dystrophy or until, in the clinical judgement of the treating physician, the patient is no longer benefiting from continuation of the treatment, whichever is sooner.
Conditions of distribution	A treatment request will be sent by the treating physician to the responsible physician at least 4 weeks before the Week 48 visit of the VBP15-004 trial if possible, to avoid the need for tapering vamorolone at the end of the trial. Within 3 weeks, the responsible physician should evaluate the request. Once the responsible physician has notified the Sponsor of the approval, in most cases vamorolone may be delivered to Site Pharmacy within 7 working days. A total of 8-10 patients in Belgium are anticipated to participate in this Compassionate Use Program.
Responsible of the program	Ana de Vera, MD ana.devera@santhera.com
Modalities for the disposal	Any unused medication needs to be returned to Santhera or destroyed in an appropriate facility as soon as possible after the patient's discontinuation from the Compassionate use program. The medication delivered for an individual patient request in the context of a Compassionate Use Programme can only be used for that particular patient.
The information for registration of suspected unexpected serious adverse reactions	From Development Safety Update Report #8 (Version date 09-Jan-2023), the following have been recognized as important potential risks: • Immunosuppression • Hepatotoxicity Adrenal Insufficiency has been recognized as expected serious adverse reaction.