PATIENT AND PARENT/CARER GUIDE

LIBMELDY™▼ 2 - 10 x 10^6 cells/mL dispersion for infusion

(atidarsagene autotemcel)

Important follow-up risk minimisation information and educational guide for patients and carers of patients who have received gene therapy with Libmeldy (an autologous $CD34^+$ cell enriched population that contains haematopoietic stem and progenitor cells (HSPC) transduced *ex vivo* using a lentiviral vector encoding the human arylsulfatase A (ARSA) gene).

Read all of this leaflet carefully before your child (or you) are given this medicine because it contains important information.

▼ This medicine is subject to additional monitoring. This will allow quick identification of new safety information. By reporting side effects, you can help provide more information on the safety of this medicine. When reporting possible side effects, include the medicinal product lot number, found on the Patient Alert Card.

If your child gets any side effects, talk to their doctor or nurse. This includes any possible side effects not listed in the package leaflet. See the end of this guide for ways to report side effects.

About this guide

The information in this guide is essential to ensure effective follow-up after your child's gene therapy treatment with Libmeldy for metachromatic leukodystrophy (MLD) and describes why and how this should be carried out. Please read this guide carefully and contact your child's specialist doctor if you have any questions.

Gene therapy for MLD and risk of blood cancer

The way that the working copy of the ARSA gene is placed into your child's stem cells can potentially modify the genetic material in these cells. This may put your child at risk of leukemia or lymphoma, types of cancer that affects the white blood cells. None of the patients who have received Libmeldy in clinical trials have developed leukemia or lymphoma, but there is a small risk that this could occur in the future. It is therefore important to monitor for the symptoms of leukemia or lymphoma.

Symptoms of leukemia or lymphoma may include fever, shortness of breath, paleness, night sweats, tiredness, swollen lymph glands, frequent infections, a tendency to bleed and/or bruise easily, or tiny red or purple spots under the skin. If your child develops any of these symptoms you should contact the specialist doctor immediately and should also report this information using the contact details provided at the end of this guide.

The specialist doctor will check your child's blood for any signs of leukemia or lymphoma during the routine yearly check-ups, which should continue after treatment.

Patient alert card

After your child is treated with Libmeldy they will receive a patient alert card that will contain very important information. The patient alert card needs to be carried by the person who has received Libmeldy or by their parent / carer in order to inform any treating healthcare professionals that they have been treated with Libmeldy.

Importance of regular monitoring

A specialist doctor will follow your child's progress over time to check for the selected risks explained in this guide and any other symptoms. It might be difficult for some patients to express how they feel; do not hesitate to report any symptoms or concerns you might have to the specialist doctor.

Enrollment in a long-term follow-up study

A long-term follow-up study has been set up to follow patients treated with Libmeldy in order to better understand the long-term effects of this medicine. You will be notified of this by your child's treating doctor.

Reporting of side effects

If your child gets any side effects, talk to their doctor, or nurse. This includes any possible side effects not listed in the package leaflet.

Report side effects to HPRA Pharmacovigilance Website: www.hpra.ie

Also report side effects to:
Orchard Therapeutics
E-mail: drugsafety@orchard-tx.com

When reporting side effects include the medicinal product lot number found on the patient alert card.