PATIENT AND PARENT/CARER GUIDE

LIBMELDY® 2-10 × 10^6 cells/mL dispersion for infusion atidarsagene autotemcel ($CD34^+$ cells)

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

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.

This document is approved by The Executive Directorate of Pharmacovigilance, at SFDA.

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 leukaemia or lymphoma, types of cancer that affects the white blood cells. It is therefore important to monitor for the symptoms of leukaemia or lymphoma.

Symptoms of leukaemia 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 leukaemia 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 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.

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 the Authority Call Center: 19999, e-mail: npc.drug@sfda.gov.sa, Website: https://ade.sfda.gov.sa/

Also report side effects to: Orchard Therapeutics, e-mail: drugsafety@orchard-tx.com and Cigalah by email to drug-safety@cigalah.com.sa.

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