

## ELEVIDYS (delandistrogene moxeparvovec-rokl) FAQ

- **What is ELEVIDYS?**
  - ELEVIDYS (delandistrogene moxeparvovec-rokl) is a prescription gene therapy used to treat people at least 4 years of age with a confirmed variant in the *DMD* gene. ELEVIDYS is given as a one-time infusion into the vein.
  - ELEVIDYS is a microdystrophin gene therapy. Microdystrophin is a smaller version of the dystrophin gene that tells the body to produce a shortened dystrophin protein in an attempt to improve or preserve muscle strength.
- **Is my child eligible for ELEVIDYS?**
  - ELEVIDYS is currently approved to treat people with Duchenne muscular dystrophy who are at least 4 years of age, and do not have antibodies to AAVrh74. If you are unsure if your child is eligible to receive ELEVIDYS, call your neuromuscular specialist, who will be able to review eligibility criteria with you and discuss whether it is appropriate for your child.
  - People with certain *DMD* gene variants, including any deletion in exon 8 and/or exon 9 in the *DMD* gene, will not be able to receive ELEVIDYS due to safety concerns. Your neuromuscular specialist will be able to discuss this in more detail with you.
  - ELEVIDYS is not yet available for people younger than age 4, or who have deletions in exon 8 and/or exon 9.
- **What is the most important information I should know about ELEVIDYS?**
  - ELEVIDYS is a microdystrophin gene therapy. Microdystrophin, or a smaller version of the dystrophin gene, is delivered to the body through a viral vector (AAVrh74), which delivers it throughout the body, including to the muscle. Because the medicine is packaged in a virus, there are certain safety precautions that should be taken, including pre-screening to make sure your child does not have antibodies to the AAVrh74 viral vector. If your child has already been exposed to the AAVrh74 viral vector and has antibodies, your child will not be able to receive ELEVIDYS. Your doctor will discuss these precautions with you.
  - If your child receives ELEVIDYS, close monitoring may be required after infusion. It will be important to talk with your doctor beforehand about how often your child will need to have labs drawn & the number of follow up visits.
  - Receiving ELEVIDYS may limit your child's ability to participate in future clinical trials. Some clinical trials have excluded patients on approved exon skipping therapies and could exclude patients on approved gene therapy as well.
  - Limited data are available for ELEVIDYS treatment in people with variants in the *DMD* gene between exons 1 to 17 and exons 59 to 71. People with deletions in these regions may be at risk for immune reactions that could be serious. It is important to discuss your child's specific variant and the potential risks with your doctor.

- Given that ELEVIDYS is a new gene therapy, please take time to discuss all of the pros and cons of receiving the gene therapy with your healthcare team. You should not feel rushed to make a decision.
- **How does my child get access to ELEVIDYS?**
  - ELEVIDYS will be administered in a clinical setting that specializes in giving ELEVIDYS and similar medications. If you want to know if your child is eligible to receive ELEVIDYS and if your neuromuscular center is able to administer it, call your neuromuscular team, who will be able to provide you with the most appropriate information on next steps.
- **What if my neuromuscular team does not administer ELEVIDYS?**
  - Because ELEVIDYS requires a specialized multidisciplinary team and specific hospital processes, not all centers will be able to administer ELEVIDYS. If your child is seen at a clinic that is not a treatment site, talk to your neuromuscular team about getting a referral to a treatment center nearby.
- **How is ELEVIDYS administered?**
  - ELEVIDYS is a prescription gene therapy that is administered as a one-time infusion through the vein (IV) over 1-2 hours at a hospital that specializes in the administration of gene therapies like ELEVIDYS. The dose your child would receive of ELEVIDYS is based on your child's weight. Before receiving ELEVIDYS, your child will receive an oral dose of a corticosteroid, and will continue taking the corticosteroid for a period of time determined by your neuromuscular team to help protect the liver. The corticosteroid can be slowly decreased once your child's liver enzymes return to baseline level.
- **What are common side effects of ELEVIDYS?**
  - ELEVIDYS, and [all gene therapies](#), can have side effects. The most common side effects include nausea and vomiting, increased liver function enzymes, pyrexia (fever), and thrombocytopenia (low platelets). Other, rare side effects can also happen that your doctor will discuss with you.
  - Some people also experienced more serious side effects, including myocarditis (inflammation of the heart muscle), acute serious liver injury and immune-mediated myositis (IMM) (inflammation of the muscles).
  - The safety and side effect information provided here is not comprehensive. Talk to your doctor about all potential side effects or what to watch for.
  - For more information, view full prescribing information here: <https://www.elevidys.com/PI>
- **What precautions are needed for family members?**
  - Because AAVrh74 is found in the stool for about one month after being treated with ELEVIDYS, there are some precautions your family should take. Practice proper hand hygiene, such as hand washing, when coming into direct contact

with patient body waste. Place potentially contaminated materials that may have the patient's bodily fluids/waste in a sealable bag and dispose into regular trash. These precautions should be followed for one month after ELEVIDYS infusion.

- Speak with your neuromuscular team about precautions for AAVrh74 exposure to siblings who may be not be eligible for ELEVIDYS at this time
- **How often will I need to follow up with my doctor?**
  - Your child will need to see your doctor frequently after he receives ELEVIDYS. Weekly blood tests will be required to monitor liver enzyme levels for three months after treatment and possibly more frequently, depending on your doctor and how your child's body responds to ELEVIDYS.
- **Will insurance cover ELEVIDYS?**
  - Because it has only recently been approved, insurance companies are still evaluating their coverage policies for ELEVIDYS. If you have questions about whether your insurance covers ELEVIDYS, you can call your insurance company directly to ask about their coverage policy. Additionally, visit [SareptaAssist.com](https://www.sareptaassist.com) for individualized support. PPMD is closely tracking insurance plans and will provide updates and support as additional information becomes available.
- **What if my child is not eligible for ELEVIDYS?**
  - The label for ELEVIDYS currently excludes patients who are under 4 years of age. There are ongoing studies in these groups of people to determine if ELEVIDYS is safe and effective, at which time Sarepta may petition the FDA to expand the label, making ELEVIDYS accessible to more people.
  - ELEVIDYS is also currently not available for people with deletions in exons 8 and/or 9. If you have questions about your child's genetic variant and whether they qualify for treatment with ELEVIDYS, contact your neuromuscular physician. Additionally, PPMD has genetic counselors on staff who can help you understand your child's genetic variant and which types of treatments may be available. Email the PPMD genetic counselors at [coordinator@parentprojectmd.org](mailto:coordinator@parentprojectmd.org).
- **What clinical studies are ongoing or planned for ELEVIDYS?**
  - There have been several studies of ELEVIDYS spanning boys from age 3 to 18 years.
    - Study 101 was an open-label study that looked at 4 boys ages 4-7 years of age; this is the study that the FDA referenced for the approval of ELEVIDYS.
    - Study 102 included 20 boys ages 5 to 8 years and was double blinded, meaning that some boys received the drug and others received a placebo, before being dosed with the drug one year later.
    - Study 103 (ENDEAVOR) is ongoing and is looking at several sets of boys, including some ages 4-7 years as well as boys ages 8-18 years who are

ambulatory, boys who are non-ambulatory, boys who are 3 years old, and boys who have variants in the gene that were excluded in other studies.

- Study 301 (EMBARK) is an ongoing global, randomized, double-blind, placebo-controlled Phase 3 trial in ambulatory boys 4-7. The EMBARK study served as the post-marketing confirmatory trial for ELEVIDYS with top line results shared in late 2023.
  - Study 303 (ENVISION) is planning to include both ambulatory boys ages 8-17 years and non-ambulatory people of all ages .
  - Study 302 (ENVOL) is evaluating ELEVIDYS in a small cohort (~21 patients) of a younger population (<4 years of age).
  - A study is being planned to evaluate the use of imlifidase prior to administration of ELEVIDYS in patients with pre-existing antibodies to AAV.
- If you are interested in clinical trials or want to know more about actively recruiting trials, visit [clinicaltrials.gov](https://clinicaltrials.gov), [PPMD's Gene Therapy & Trials site](#), or reach out to one of PPMD's genetic counselors ([coordinator@parentprojectmd.org](mailto:coordinator@parentprojectmd.org)).

- **How many people have received ELEVIDYS?**

- As of June 2023, more than 140 individuals with Duchenne have been dosed with ELEVIDYS.

- **What does Traditional Approval mean?**

- ELEVIDYS was originally approved utilizing the Accelerated Approval pathway on the surrogate outcome measure of dystrophin production and the assessment by regulatory experts that dystrophin production in a disease caused by its absence is 'reasonably likely to have clinical benefit.' After review of Sarepta Therapeutics' efficacy supplement, on June 20, 2024, FDA converted the ELEVIDYS accelerated approval to a traditional approval for ambulatory patients.
- ELEVIDYS is approved for non-ambulatory patients through the Accelerated Approval pathway based on the surrogate outcome of micro-dystrophin expression. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.
- Traditional approval from the FDA is based on trial data that shows a drug will provide meaningful benefit or clinical outcome. A drug that has received Accelerated Approval is based on a surrogate predicting it *is likely* to provide meaningful benefits that outweigh its known and potential risks for the intended population.
- For eligible patients and families, there should not be a meaningful difference between the two approval pathways, but we have seen insurance policies exclude coverage to accelerated approval therapies and we hope the full approval will reduce barriers to patient access.

- **What does Accelerated Approval mean?**
  - The FDA Accelerated Approval Program allows for products that meet regulatory rigor based on efficacy of a surrogate endpoint that is reasonably likely to have clinical benefit for select patient communities in which there is significant unmet need.
  - By definition, limited clinical data for products approved through accelerated approval will exist at the time of approval and in early stages of the Phase 4 environment. In these circumstances, this lack of clinical data is not a reflection of the robustness of the therapy, but rather the regulatory review pathway agreed upon by the product developer and the FDA.
  - ELEVIDYS is now an approved therapy and is no longer considered an investigational product.
  - ELEVIDYS was originally approved utilizing the Accelerated Approval pathway on the surrogate outcome measure of dystrophin production and the assessment by regulatory experts that dystrophin production in a disease caused by its absence is ‘reasonably likely to have clinical benefit.’ For non-ambulatory patients, ELEVIDYS is still considered an approved therapy under the Accelerated Approval pathway
  - This demonstration of clinical benefit will depend upon product availability to amenable patients enabling the collection of data on long term outcomes. [PPMD and the Duchenne clinical expert community](#)—as convened by the leaders of the Certified Duchenne Care Centers—assert that coverage of a drug must reflect the FDA approval status of a drug.
  
- **Where can I get more answers to my questions about ELEVIDYS?**
  - If you have more questions about ELEVIDYS, talk to your neuromuscular provider. If you have nonmedical questions about ELEVIDYS, you can visit [Elevidys.com](#) or [SareptAssist.com](#).
  - If you have general questions about approved or investigational therapies in Duchenne and whether your child is eligible, contact the PPMD Genetic Counselors at [coordinator@parentprojectmd.org](mailto:coordinator@parentprojectmd.org).
  - If you have questions about access, navigating care, or are looking for other types of individualized support, connect with the PPMD Team directly through [PPMD for You](#).

If you have questions about accessing one of Sarepta’s approved therapies and you live inside the United States please contact SareptAssist by calling 1-888-SAREPTA (1-888-727-3782), or emailing [sareptassist@Sarepta.com](mailto:sareptassist@Sarepta.com). More information about SareptAssist may be found here: <https://www.sarepta.com/sareptassist>