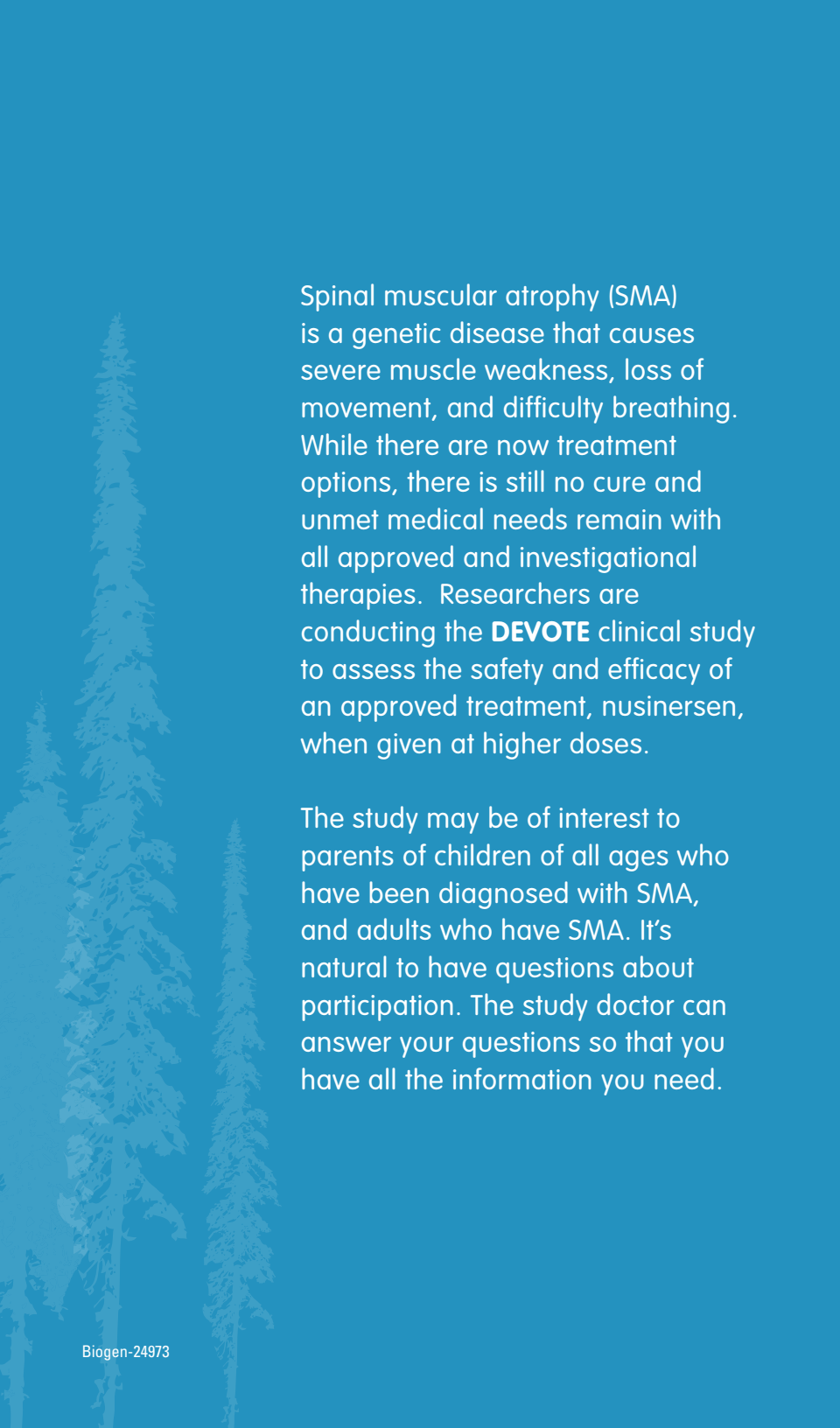


The **DEVOTE** Study

A Phase 2/3 Clinical Study of
Nusinersen in Infants, Children, and
Adults with Spinal Muscular Atrophy



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Spinal muscular atrophy (SMA) is a genetic disease that causes severe muscle weakness, loss of movement, and difficulty breathing. While there are now treatment options, there is still no cure and unmet medical needs remain with all approved and investigational therapies. Researchers are conducting the **DEVOTE** clinical study to assess the safety and efficacy of an approved treatment, nusinersen, when given at higher doses.

The study may be of interest to parents of children of all ages who have been diagnosed with SMA, and adults who have SMA. It's natural to have questions about participation. The study doctor can answer your questions so that you have all the information you need.

About Spinal Muscular Atrophy (SMA)

SMA affects approximately 1 out of every 10,000 babies. It is an autosomal recessive genetic disorder, which means that generally both parents must carry a mutated survival motor neuron 1 (SMN1) gene for their baby to inherit SMA. Parents who do not show symptoms of SMA are considered carriers because each parent has one mutated copy of the SMN1 gene and two mutated copies are required to have the disease.

Clinical studies, like this one, are conducted to answer questions about the disease and investigational approaches, providing information that may help with the development of future medical treatments.



About the Study Drug

SMA is caused by a mutation, or change, in the SMN1 gene. This gene produces a protein called survival motor neuron (SMN) that is important for nerve and muscle function. Individuals with SMA do not produce enough of this protein. There is a “back-up” gene called SMN2 that produces a small amount of the SMN protein; however, the SMN2 gene cannot fully make up for the mutated SMN1 gene.

The study drug, nusinersen, works by increasing the amount of the SMN protein coming from the SMN2 gene to help nerves and muscles function properly. The **DEVOTE** study is evaluating nusinersen’s safety and efficacy when given at higher doses.



Administration of the Study Drug

All study participants will receive the study drug over a period of approximately 10 months. It is administered through an injection, using a thin needle, into a fluid-filled space at the base of the spinal cord (in the lower back). This type of procedure is called a lumbar puncture.

The study is for infants, children, and adults* of all ages who are genetically diagnosed** with SMA.

* Adults 18 years and older must be ambulatory.

**A genetic diagnosis is determined by a genetic test. This is a type of medical test that can determine whether a person carries genes for a specific inherited disease. To be eligible for this study, a genetic test must confirm that the potential participant has inherited the gene that causes SMA. A genetic test can be conducted using a small amount of blood.





Starting the Conversation

If you are interested in the **DEVOTE** study, you will be given an opportunity to ask the study doctor questions about the study procedures and requirements. You will be provided with an Informed Consent Form that explains the details of the study.

If you decide you or your child would like to participate, you must provide your consent by signing the form. This process is called the informed consent process, and it is a standard process for participation in a clinical study.



For You and Your Family: What Participation Means

Once you have completed the informed consent process, the study doctor and the study staff will conduct medical tests and assessments to determine if you or your child (the participant) is eligible to participate.

If the participant is eligible and chooses to participate, participation, including screening and follow-up visits, will last up to 11 months. During this time, there are between five and nine visits to the study location. At most visits, the participant will be asked to stay overnight at the study center for up to 24 hours.

The study staff will also call between certain visits to check on the participant's health, and ask if they have started any new medications or had any changes in respiratory status.

Neither you nor your insurance provider will be charged for the costs of any procedures performed as part of the clinical study.



All study participants will be provided with the following at no cost:

- nusinersen, the study drug
- study-related care and monitoring
- appointments with the study doctor

At the end of the study, participants may be eligible to participate in an extension study and continue to receive the study drug at no cost.

Participants will remain in the care of their regular doctors while participating in the **DEVOTE** study for routine office visits and checkups.

Ask us about travel support:

Travel support may be available in the form of coordination and coverage of expenses. (Depending on your distance to the study location, this may include ground transportation, flight, and hotel.)



About Clinical Studies

Clinical studies play an important part in the development of medical treatments. They are conducted to test investigational drugs and therapies, or in this case, a higher dose of a currently approved therapy, to determine if they are safe and effective. Participation is always voluntary, and you can decide to end participation at any time and for any reason.

