

A Clinical Study Evaluating a Gene Therapy for Rett Syndrome

About the Investigational Gene Therapy

Gene therapy is designed to deliver a working copy of a gene to a person's body to treat a genetic disorder like Rett syndrome. The investigational gene therapy being evaluated in Embolden™ is called NGN-401.

NGN-401 is designed to deliver an *MECP2* gene to make a fully working MeCP2 protein. Having too little MeCP2 protein causes Rett Syndrome. Too much MeCP2 protein can be harmful, so NGN-401 is designed to prevent producing too much MeCP2 protein.

NGN-401 is delivered by an adeno-associated virus (AAV) to the brain through a one-time surgical procedure. Some viruses, such as AAV, are good at delivering genetic material like NGN-401 to cells in the body. When a virus is used in this way, it does not cause disease.

What to Expect



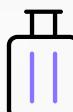
A lot of caregiver time, tasks required

100 %

All participants receive the investigational gene therapy



First 3 months must stay within 2 hours of study site



Short-term relocation to be near a study site may be needed



First 5+ days in hospital



3 years of study visits



No study-related or travel costs



Many tests and procedures to monitor your daughter's health



12-year follow-up study participation expected



Participating In a Clinical Trial is Voluntary

There are risks to participating in any clinical study. Your daughter may or may not benefit from this research. Talk to your doctor to learn more about Embolden and help you decide if it is the right choice for your family.