Why is the STR1VE study important?

Pharmaceutical companies are required to conduct research studies to learn more about experimental treatments/drugs before they are made available to the public. The results of this study will provide more information about the safety and effectiveness of the experimental treatment. By taking part in the STR1VE study, you and your baby will play an important role in the future of SMA Type 1 treatments.

To learn more, please visit studysmanow.com or contact:
Who is eligible to participate in the STR1VE study?

To pre-qualify, your baby must be:

- Younger than 6 months of age
- Diagnosed with SMA Type 1
- Up to date on his/her vaccinations

All study-related visits, tests, and treatments will be provided to participants at no cost. In addition, reimbursement for study-related travel may be provided.

What will happen during the STR1VE study?

If your baby is eligible for this study, and you agree to allow him or her to participate, they will receive one infusion of the experimental treatment. All babies enrolled in this study will receive only one infusion of the experimental treatment.

Your baby will receive the infusion at the study site and will stay overnight and up to 48 hours for observation and evaluation. After your baby is released from the hospital, you will be asked to bring him or her to follow-up visits for the remainder of the study so that doctors and study staff can evaluate your baby’s health.

Four of these visits will take place in the month after the infusion, and will be followed by monthly visits until your baby reaches 18 months of age. Depending on how old your baby is when he or she begins the study, your baby’s total study participation will range from 13 months to 17 months.

What are the risks and benefits related to the STR1VE study?

As with any research study, improvement of your baby’s SMA Type 1 cannot be guaranteed. It is possible your baby could experience a side effect while in this study, but at this time, not all effects (good and bad) of the experimental treatment are known.

The study staff will provide you with a complete list of known risks related to this study.

Because research studies can affect the health and safety of participants, your baby will be closely monitored during this study. Researchers for the STR1VE study designed a protocol, which explains all study procedures in complete detail. An independent review board responsible for participant safety reviewed and approved this protocol and requires that it be followed exactly.

What if I have questions?

The STR1VE study staff is always available to answer any questions or concerns you may have about the study, experimental treatment, and infusion.