Life After Gene Therapy

For sound, stream audio through your speakers. If you are having trouble accessing sound, please send a message using the chat box in the lower left hand corner.

 Alone we are rare. Together we are strong.
This webinar is being recorded.
Submit your questions using the chat function. It can be found at the left hand side of the window.
NORD, an independent nonprofit, is leading the fight to improve the lives of rare disease patients and families.

We do this by supporting patients and organizations, accelerating research, providing education, disseminating information and driving public policy.
Notes & Updates

Learn more
https://rarediseases.org/living-rare-forum/
Speakers

Charles Hough
Gene Therapy Patient
Sickle Cell Disease

Nicole Almeida
Mother of Gene Therapy Patient
Spinal Muscular Atrophy
Gene Therapy for Sickle Cell Disease

Charles Hough
Symptoms
My Path to the Trial
My preparations involved...
Bi-weekly Blood Draws
For about 1 year and a half

To the left, NIH will remove 250ml of blood from my body. Then right after I will receive two pints of fresh blood.
Stem Cell Collection
Chemotherapy
The Road to Recovery
I’m Going Home!
Hyperpigmentation
5 months past chemo!
Hinagdaanan Cave, Bohol Philippines
Panglao Philippines
Gene Therapy for SMA

Nicole Almeida
RESULTS:  SMN1 copy number:  0 (See Interpretation)
SMN2 copy number:  2 (See Comments)

INTERPRETATION:
This fetus has no copies of SMN1 and is therefore predicted to be affected with spinal muscular atrophy, a disease of variable age of onset and severity. Genetic counseling is recommended.
Gene Therapy-Infusion Day: August 6, 2015
Gene Therapy: Trial Follow-ups
Matteo’s Amazing Doctors
Milestones and Progress

1st Birthday

2nd Birthday

3rd Birthday

4th Birthday
Adventures
Adventures and Traveling
The Future
Question and Answer Session
Resources for Patients and Caregivers

Clinical Trials
Clinicaltrials.gov or asgct.org/clinicaltrials

National Institutes of Health

Food and Drug Administration
https://www.fda.gov/vaccines-blood-biologics/cellular-gene-therapy-products

ASGCT
https://www.asgct.org/education

NORD
https://rarediseases.org/video-topic/research-science/#watch-39678
Questions?

Submit your questions in the chat box.

Nicole and Charles will answer them in the order in which they came in and based on relevance to the discussion.
Thank you.

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