



Life After Gene Therapy

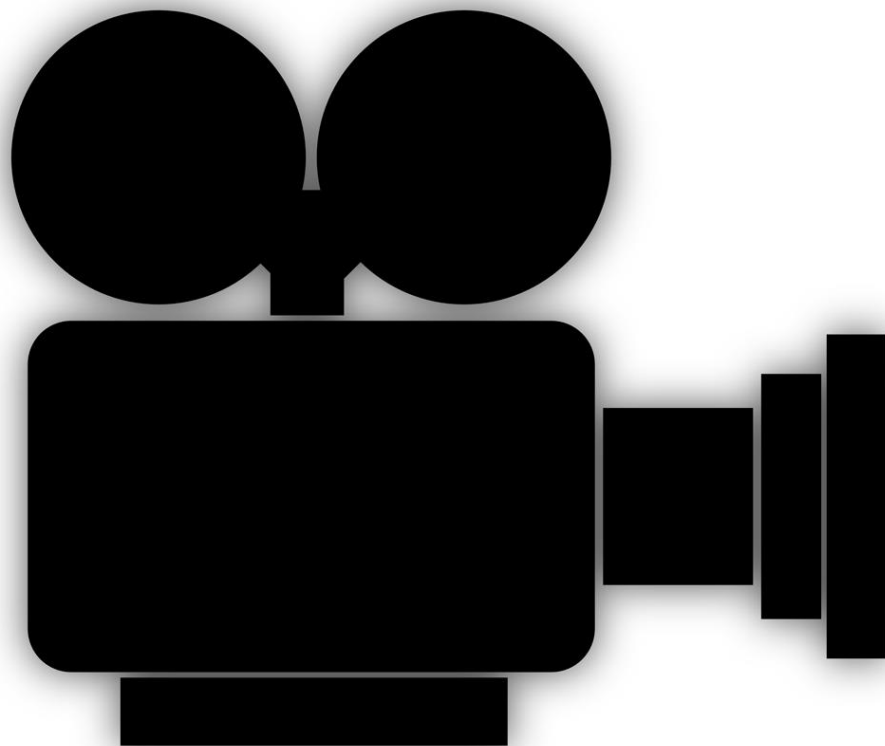
For sound, stream audio through your speakers.

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.®

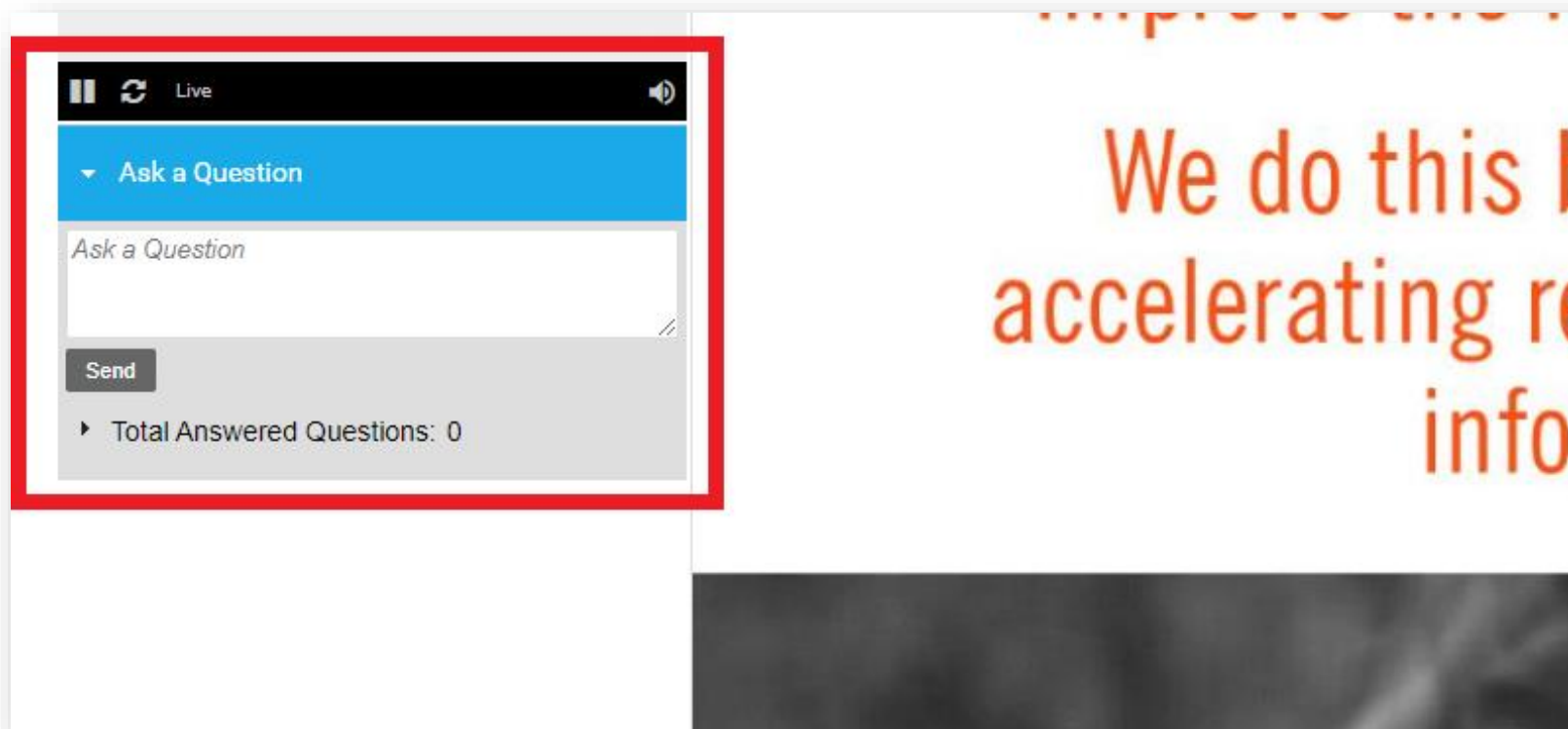


This webinar is being recorded.



Question and Answer Session

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.



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Notes & Updates



Learn more

<https://rarediseases.org/living-rare-forum/>



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Speakers



Charles Hough
Gene Therapy Patient
Sickle Cell Disease



Nicole Almeida
Mother of Gene Therapy Patient
Spinal Muscular Atrophy



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Gene Therapy for Sickle Cell Disease

Charles Hough



Vaughn family: Son, Morgan (left), diagnosed with Necrotizing Enterocolitis at four days old

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Symptoms



My Path to the Trial



My preparations involved...



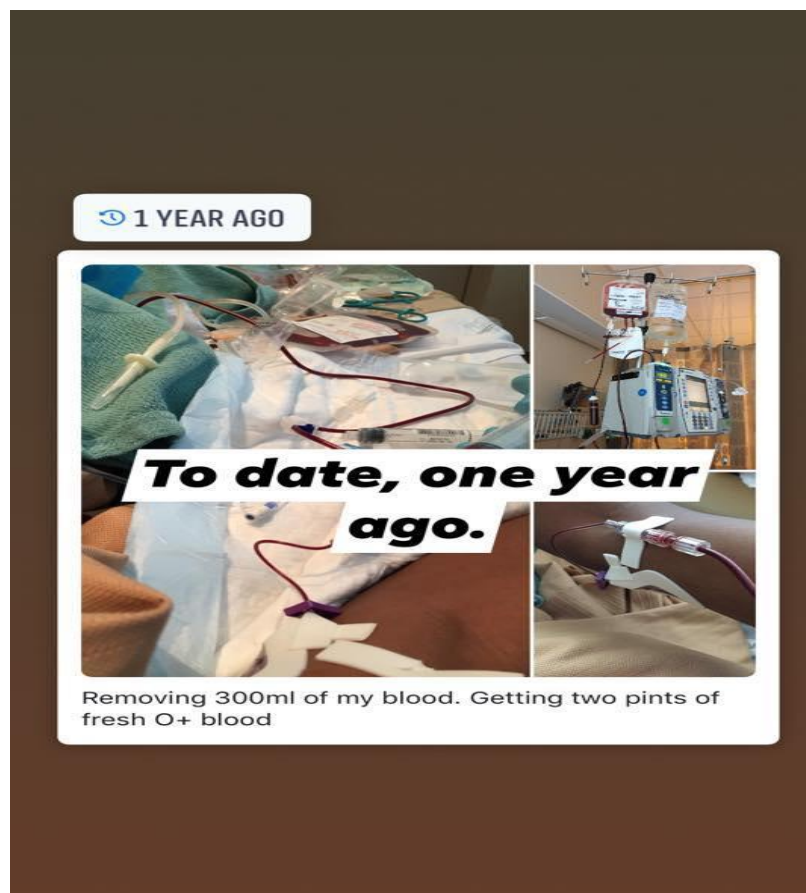
Bi-weekly Blood Draws



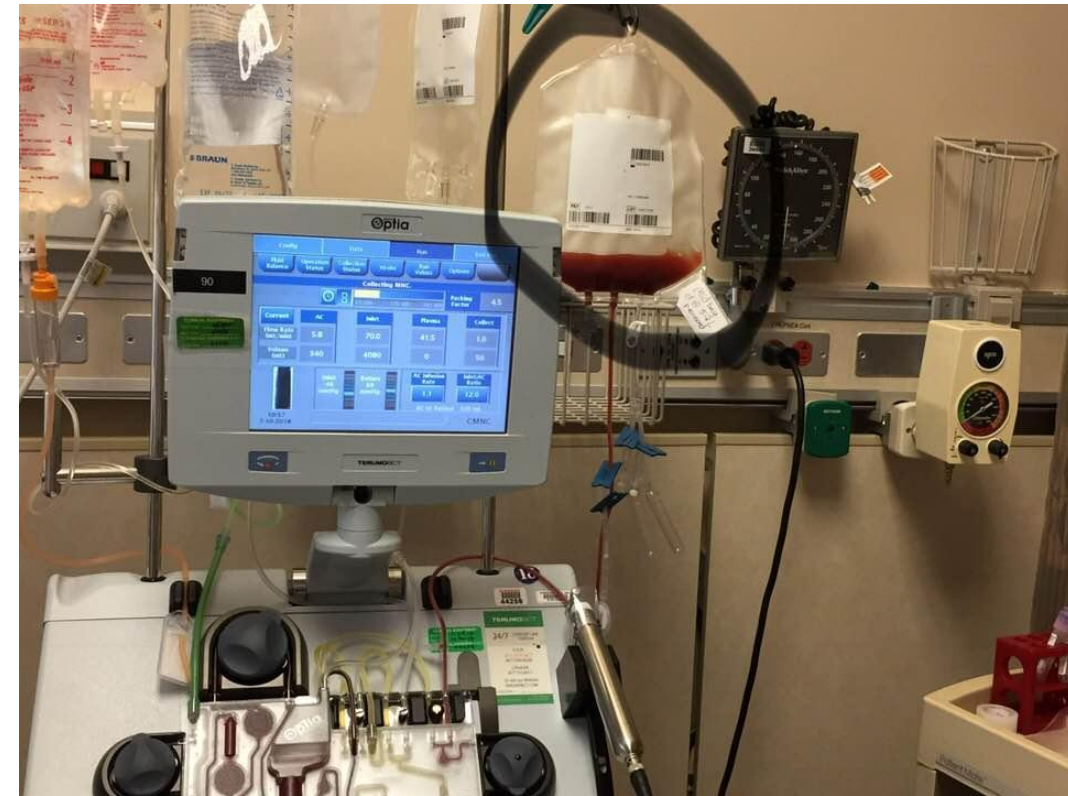
For about 1 year and a half



To the left ,NIH will remove 250ml of blood from my body. Then right after I will receive two pints of fresh blood.



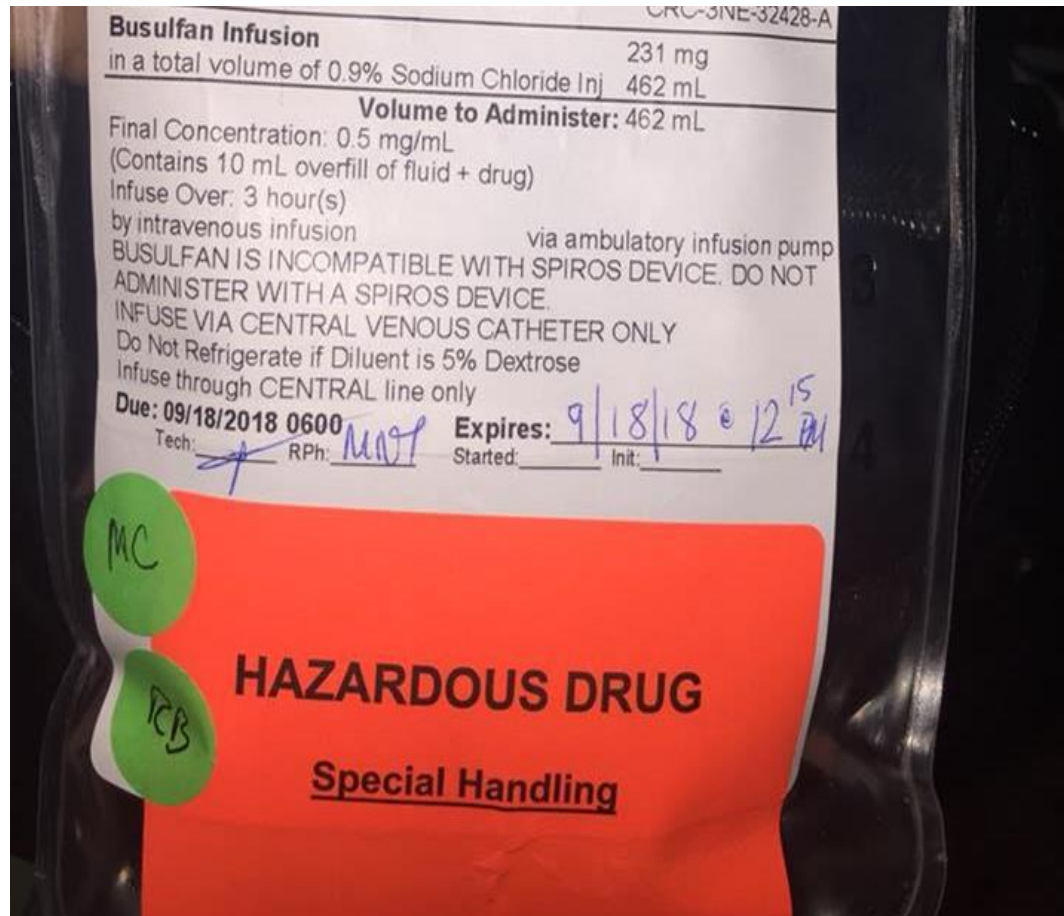
Stem Cell Collection



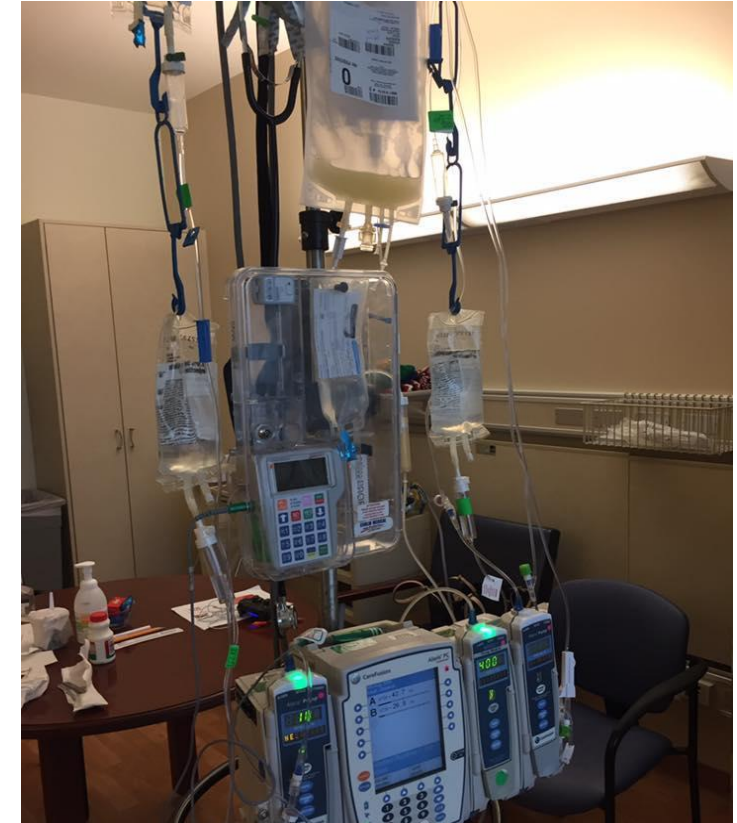
My Room in NIH



Chemotherapy



After Chemo Was Administered



The Road to Recovery



I'm Going Home!

Monocytes.	1.0
Eosinophils	24.2
Basophils	0.0
Neutrophil Absolute	0.51
Lymphocyte Absolute	2.00
Monocyte Absolute	0.81
Eosinophil Absolute	0.00



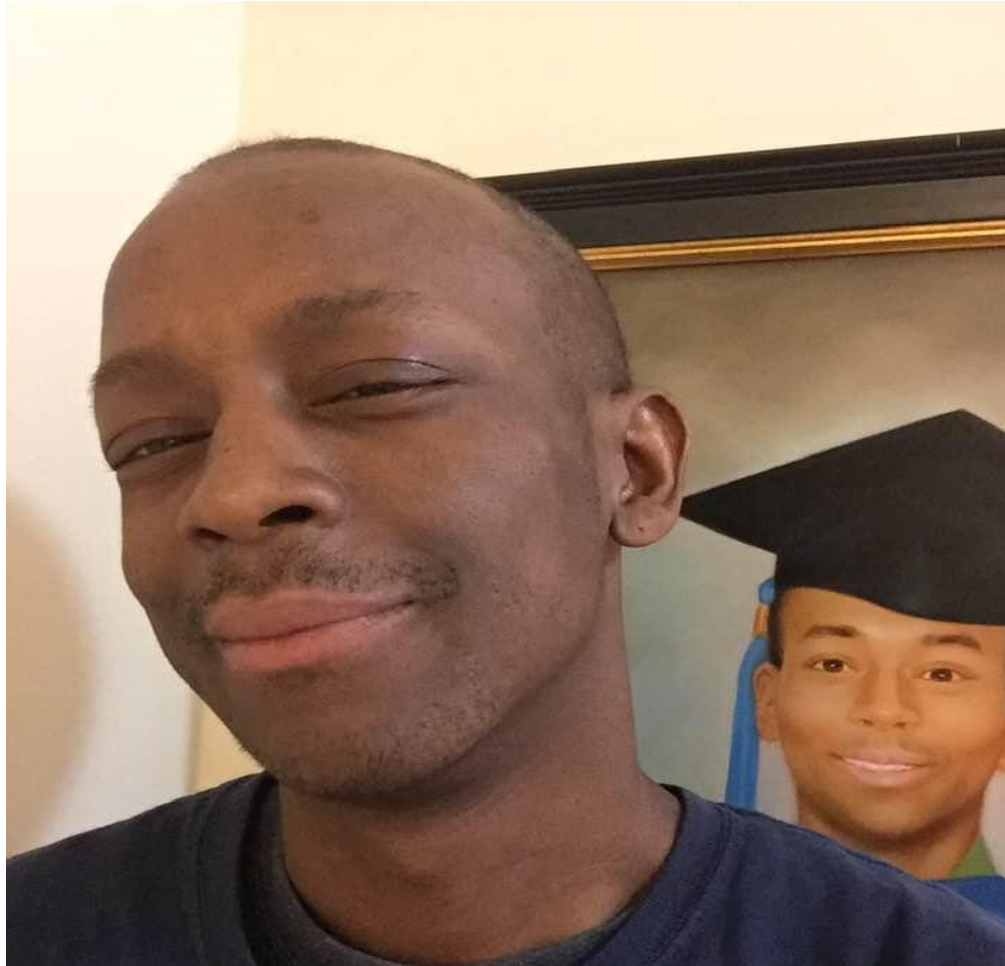
Medications



Hyperpigmentation



5 months past chemo!



Hinagdaanan Cave, Bohol Philippines



Panglao Philippines



Gene Therapy for SMA

Nicole Almeida



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Madilyn Yang (far left) has been battling a rare breathing disorder since birth called Central Congenital Hypoventilation Syndrome (CCHS) or Ondine's Curse.

Pregnancy and Diagnosis



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



Adventures



The Future





Question and Answer Session



Vaughn family: Son, Morgan (left), diagnosed with Necrotizing Enterocolitis at four days old

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Resources for Patients and Caregivers

Clinical Trials

[Clinicaltrials.gov](https://clinicaltrials.gov) or asgct.org/clinicaltrials

National Institutes of Health

<https://ghr.nlm.nih.gov/primer/therapy/genetherapy>

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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