AskBio™

The AAvenger™

ADENO-ASSOCIATED VIRUS (AAV) GENE THERAPY NEWS

SUMMER 2020 | VOL.2

Jazz Pratt,
Research Associate

ASKBIO: MOVING FORWARD, OVERCOMING OBSTACLES

As the scientific community works to find vaccines and treatments for COVID-19, we cannot lose sight of patients around the world whose futures depend on new gene therapies. At AskBio, we are using this time while research sites are seeing fewer patients to accelerate the administrative work that will allow us to quickly initiate new studies once operations start ramping up again.

We are also amending and redesigning protocols for our planned and ongoing studies in collaboration with patient advocates to allow patients to complete as many visits at home as possible — taking advantage of local resources and technology to limit travel. It is our hope that these changes will create long-term benefits for patients and make gene therapy studies an option for more patients for whom travel is difficult.

MEET THE TEAM: DR. SAM HOPKINS – PROGRAM LEADER, POMPE DISEASE

Dr. Hopkins is a scientist at AskBio and has over 35 years of experience in the pharmaceutical industry. He is responsible for managing our clinical research and development program and evaluating AAV-based gene therapy vectors for the treatment of rare neuromuscular diseases, with our lead indication being Pompe disease.

Throughout my career I've been involved in many aspects of research including discovery, non-clinical research and all phases of clinical development, but it's the potential to make a life-changing impact in patients' lives that drew me to AskBio.

FACES OF HOPE

We have an inspirational program called *Faces of Hope* where brave individuals and families welcome us into their lives to share a glimpse of their journey living with a genetic disease. These *Faces of Hope* help ground us, inspire us and remind us all why we continue to push forward every day with our best efforts.

Earlier this year, we were honored to have Dakota, one of the stars of *Faces of Hope*, and his mother, Deana, tour our lab and meet most of our NC-based staff. It was during this inaugural celebration of the program that he helped with the unveiling of our other stars: Princeton, Sammy and Lisa.

We can only achieve a new future with their faces, voices and stories in mind. Patients and families are critical partners in the search to better understand genetic disorders and develop breakthrough therapies.

Here, they have given us the opportunity to bring their voice into our work, so that they can be heard, embraced and influence our decision making. What we learn from our patient-collaborators and their families greatly informs our paths to clinical development. Together, we can aspire to obtain meaningful outcomes for individuals and families. We are so honored that these families have been willing to share their stories, perspective, journey and homes with us.

Matt Alsante, Dakota Bridges, Dr. Jude Samulski, Sheila Mikhail

Amanda Hobbs, Research Associate II

WHAT IS GENE THERAPY?

Genes are made up of DNA and act as the blueprints of the human body. They instruct the body's cells how to make proteins critical to their function and other important hereditary factors. Sometimes, people are born with or acquire errors in their genes that prevent them from making an important protein, which leads to a disease.

Gene therapy is a technique that uses genes to treat or prevent disease. This technique can allow doctors to treat a disorder by inserting a gene into a patient's cells. This approach is different from traditional drug-based approaches, which may treat symptoms but not the underlying genetic problems.

Gene therapy uses modified viral particles or other technologies to deliver therapeutic genes to cells in a specific part of your body to address genetic diseases at their source. Treatment can replace a faulty gene, introduce a new gene whose function is to cure or favorably modify the clinical course of a condition, or inactivate or "knock out" a faulty gene that is not functioning properly.

AskBio uses one of the most exciting advances in modern medicine, the adeno-associated virus (AAV), as an effective delivery system for therapeutic genetic material into living tissue. AAV gene therapy has broad therapeutic implications for a vast array of diseases, with the hope of improving the quality of life for patients.

CLINICAL TRIAL UPDATES

Sudhanthira Shanmugam, Scientist II

LIMB-GIRDLE MUSCULAR DYSTROPHY 2I/R9

Enrolling Our First Clinical Study this Winter

Based on encouraging pre-clinical animal models, we are planning to conduct a clinical trial of a gene therapy, LION-101, for limb-girdle muscular dystrophy 2i/R9 starting in early 2021.

We have developed LION-101 as a strategy to make up for the damaged, non-functioning gene, FKRP. We provide a correct copy of the FKRP gene using the shell of a harmless virus that has had the viral DNA removed. The gene is delivered to the voluntary and heart muscles, where it is expected to begin working like a non-damaged FKRP gene.

To limit hospital visits during this time of COVID-19, we are going to arrange as many in-home visits as are possible after the initial treatment. Please visit our website at *www.askbio.com/genetic-disease-focus/limb-girdle* for updates as they become available.

POMPE DISEASE CLINICAL STUDY

ClinicalTrails.gov Identifier: NCT03533673

We are currently enrolling adult subjects with late-onset Pompe disease (LOPD) to assess multiple doses of the gene ACT-101. The current standard of care for Pompe disease involves patients receiving an infusion of the enzyme they cannot make (enzyme replacement therapy or ERT). ACT-101 is a designed to deliver a functioning copy of the GAA gene (malfunctioning in Pompe disease) to the liver of subject, so that their own production of GAA is restored to a level sufficient enough to no longer require ERT. Initial enrollment is complete, and we are expanding enrollment through a protocol amendment.

Dr. Josh Grieger,
Chief Technology Officer

CONGESTIVE HEART FAILURE

ClinicalTrails.gov Identifier: NCT04179643

We are actively enrolling patients with NYHA Class III heart failure to assess three doses of NAN-101. NAN-101 is a gene therapy that aims to halt the progression of congestive heart failure.

The primary objective of this study is to assess the safety of NAN-101 for the treatment of NYHA Class III heart failure, as well as assess the impact of this treatment on patient health as measured by changes in exercise capacity, heart function and other factors including quality of life.

For more information, please visit *www.clinicaltrials.gov* or email us at *askfirst@askbio.com*.

HOPE TRAVELS: CELEBRATING THE 4TH OF JULY

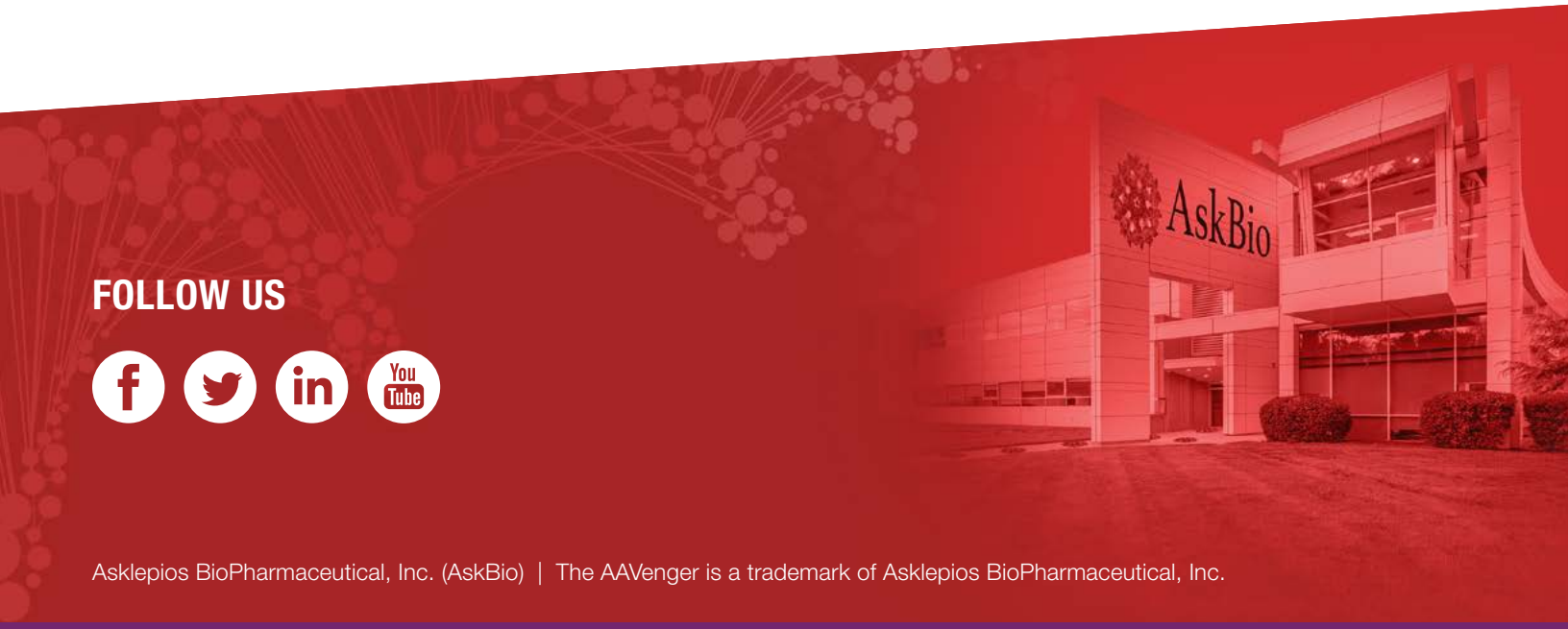
Hope the Sloth has been adopted as the unofficial mascot of the Pompe community. In addition to studying gene therapy at AskBio, she is helping to celebrate our Independence Day with her big brother Alfie. As Hope continues to travel throughout our labs and the world, look forward to more updates!

NEWSLETTER

Sign up to get our latest patient news at *www.askbio.com/patient-advocacy*

LET'S TALK

Email us at *askfirst@askbio.com*



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