DEAR STAR FAMILIES AND SUPPORTERS:

As we find ourselves in these unprecedented times, we are delighted to update all of you on our progress.

Yes, PROGRESS!

We have been very successful in 2020.

Amidst the chaos, concern, and uncertainty of COVID-19, we wanted to extend to you all the hope, ambition, and unwavering optimism that STAR sees in the future.

Many exciting events have unfolded and we are delighted to share with you our latest developments.
Recently, STAR submitted an $80,000 payment for the NIH to continue its endeavors with the National Human Genome Research Institute. Our lab technician, Mary Hackbarth, will continue to work under the direction and guidance of Dr. William Gahl, Dr. Marjan Huizing and Dr. David Adams. We are grateful for the continued partnership with these esteemed doctors and researchers.

Update on Salla disease-related research activities at NIH

Report from Mary Hackbarth, Marjan Huizing Ph.D., and William Gahl M.D., Ph.D.
May 20, 2020

NIH has been operating on maximum teleworking capabilities since mid-March. That means that while many researchers, including those who work in the Gahl lab at the National Human Genome Research Institute (NHGRI) at NIH, have been unable to go into the physical lab spaces and conduct new experiments, we have had time to analyze data from previous experiments, take online courses to better inform our research plans, and review the literature while organizing experiments to start up as soon as we are able. Also, thanks to the essential employees and dedicated veterinary and scientific staff at the NHGRI, both within the Gahl group and in our animal core facilities, our Salla mouse colony is being maintained.

The Gahl lab plans to hold a virtual scientific workshop to discuss Salla research with members of the SASD Consortium and our collaborators around the world sometime this fall. Although members of the community will not be able to meet in person for the workshop as originally planned, we hope that this virtual meeting will allow many investigators to participate in the scientific discussion, perhaps at a time when lab work at NIH and around the world may be ramping back up to full steam.

NIH hopes to schedule an in-person meeting with scientists and STAR families when it is safe to do so.
NEW FUNDING FOR RESEARCH AT THE EINSTEIN COLLEGE OF MEDICINE

As many of you know, research at the Albert Einstein College of Medicine, under the direction of Dr. Steven U. Walkley and Dr. Kostatine Dobrenis, has demonstrated promising indicators with the drug Miglustat. Due to the COVID-19 crisis, opportunities to secure scientific grants to continue this research on Salla have been limited. The STAR Board of Directors determined to allocate $25,000 toward this important research to ensure this fruitful work in funded through 2021. This gift was made possible by the generosity of the Kaiser family in Switzerland. Thank you, Kaiser Family!

Additionally, our contracts with the NIH and Einstein are being altered to allow for time and science lost during the shutdown. We graciously appreciate the flexibility and partnership with these fantastic institutions!

As we look ahead to 2021, STAR is financially healthy, thanks to all of the STAR families and supporters who have raised money and contributed to the STAR Foundation.

thank you
EXPANDING OUR NETWORK OF RESEARCH PARTNERS

In February, STAR representatives attended the World Symposium of Rare Diseases in Orlando, Florida. At this conference, a research group from the Children’s Hospital of Orange County, California, (CHOC) won a “Young Investigators” Award for its research on Pompe disease leveraging various gene editing techniques through the Center for Advancing Rare Disease Editing (CARE). STAR was approached by the CHOC team, led by Dr. Raymond Wang, to gauge our interest in forming a partnership. Based on the guidance of our Scientific Advisory Board, led by Dr. Steven Walkley, Dr. Melissa Wasserstein, and Dr. William Gahl, we have integrated CHOC’s efforts to the ongoing research at the NIH and Einstein College of Medicine. We are thrilled to have three preeminent institutions now collaborating, sharing cell models, mouse models, and other knowledge to fast track Salla research! Our expanded research team from CHOC now includes:

Jerry Harb, MS, Research Associate, CHOC/CARE  
Dr. Jefferey Y. Huang, Ph.D., Founder and Senior Scientist, CHOC Center for Advancing Rare Disease Editing (CARE)  
Dr. Shih-Shin Kan, Ph.D., Scientist II at CHOC  
Dr. Raymond Wang, M.D., Director, CHOC Multidisciplinary Lysosomal Storage Disorder Program
"STAR FOR MARTINA" FIELD DAY A RESOUNDING SUCCESS

Salla Treatment And Research would like to give a special thanks to Jose and Julissa Actis. On February 29, Rare Disease Day, they held a massive event in Santo Domingo, Dominican Republic in honor of their daughter, Martina. Their “Field Day” raised $80,000, enabling STAR to fund further research at the NIH! We are so fortunate for this incredible contribution to our Foundation. Actis Family, words will never fully express how thankful we all are for your friendship and shared commitment in finding a treatment for Salla.

WATCH THE "STAR FOR MARTINA FIELD DAY" VIDEOS AT WWW.SALLARESEARCH.ORG
SALLA TREATMENT AND RESEARCH OFFICIALLY GOES INTERNATIONAL

STAR is officially international! We now have Board Members representing Europe and Latin America! In April, we welcomed Jose Actis of the Dominican Republic (daughter, Martina, age 3) and Kathrine Roa of Norway (daughter, Anea, age 7) to the STAR Board of Directors.

This month, Ann Persson from Sweden (son, Elis, age 9) showcased the STAR Foundation at this year’s European Conference on Rare Diseases (ECRD-30). Scheduled to take place in Stockholm, it was held virtually due to the COVID-19 pandemic. Ann worked tirelessly, creating a poster and presentation with the help of Mary Hackbarth and Caroline Moberg (son, Johan, age 8). We are thrilled that Ann earned 11th place out of hundreds of participants! We are certain this will garner the attention of more doctors, families, and supporters throughout Europe and Scandinavia.

Thank you Ann!

The use of social media to create a foundation and drive research on rare diseases

Ann Persson, STAR foundation

Introduction – S.T.A.R
S.T.A.R - Salla Treatment and Research Foundation (S.T.A.R) was established by the Foglio family in New York in 2018 to promote research, treatments, awareness, and family networks for Salla disease. S.T.A.R is driven by an unwavering belief that with sufficient support, a small team of committed researchers, families, supporters, and advocates can create meaningful solutions for those impacted by the disease.

Salla disease
Salla disease, intermediate severe Salla disease, and irritant free sialic acid storage disease (ISSD) are neurodegenerative disorders resulting from increased lysosomal storage of free sialic acid. Salla disease has been reported in approximately 200 individuals in the world, mainly from Finland and Sweden.

Social media to unite families from all over the world
Through social media, STAR has managed to connect about 30 families in 13 countries around the world. A network for parents has been created where they can support each other, share knowledge and experience on how to further develop their child.

Funding Events
• Private funding initiatives using social media such as Facebook
• Auctions, concerts, and gala
• An international walk held in 3 countries arranged and informed mostly through social media.
• In less than two year these initiatives have been able to raise funding for at least 2 years of research at NIH, US.

Research
Below are just a few examples on research conducted with the support from S.T.A.R;
• A knockout mouse model of Salla disease established.
• A new Salla disease knockin mouse model is being generated at NIH, US.
• SLC17A5/Salln mutation analysis allowing for research confirmation of diagnosis has been established at NIH, US.
• The Salla disease intracellular phenotype is being characterized at NIH, US for use in drug screening/testing + purpose.
FROM OUR NEW BOARD MEMBER, KATHRINE ROA
STAR NORWAY

I’m Kathrine. I am married to Stian and we have two wonderful children, Jonas at 10 years and Anea at 6 years. We live right outside Hønefoss, Norway. Anea has Salla disease, diagnosed when she was 2.5 years old, but we started our journey when she was 4 months old. **We are committed to spreading knowledge so that families will not need to experience the same feeling that we did when receiving this diagnosis, with so little information and hope.** We want to do something about this!

At the end of January 2020, a 4-day family course was held for all families in Norway with Salla at FRAMBU, which is a competence center for rare diseases here in Norway. Prior to this course we received a request to provide a lecture to discuss our journey with Salla, both good and bad. Two doctors from Finland also conducted a lecture, Dr. Liisa Paavola, Ph.D, a specialist in neuropsychology, and Tarja Varho, MD, a specialist in pediatric neurology. It was very exciting and educational to have a meeting with them and to create more useful and stronger contacts for the future.

**Stian and I have now started a separate organization in Norway, STAR Norway (Salla Treatment And Research Foundation).** This is exciting! One separate chapter in Norway with the support of STAR and Jessica. The road continues to grow as we move forward and we learn something new each day. We seek to build a network for families around the world, advancing awareness and knowledge about the disease. With the huge support we are already receiving, I am confident that together, we can do this!

Kathrine
The Foglio family shared the STAR Foundation’s story with families of another rare disease, KDM5C, at Einstein College of Medicine.

The Actis family organized the highest-grossing fundraiser yet for Salla disease during the “Star for Martina” Field Day in Santo Domingo, Dominican Republic.

STAR scientific advisor Dr. Steven U. Walkley gave a lecture on rare disease collaboration between researchers and families.

Ann Persson virtually showcased the STAR Foundation at the European Conference on Rare Diseases.

STAR scientific advisor Dr. Melissa Wasserstein with Ben, age 5, at the Einstein College of Medicine.
YOUR SUPPORT

We remain committed to continuing the research against Salla and continue to support efforts at the National Institute of Health, Einstein College, Children’s Hospital of Orange County, California, and several other key partners all working in lockstep based on the guidance of our Scientific Advisory Board. It is with tremendous pride and gratitude to you all that we are able to secure another year of research with these partners. From the bottom of our hearts and on behalf of all parents and families impacted by Salla, you have our gratitude and our thanks.

It is impossible to ignore the impacts of COVID-19; many of the friends and families of the Board and the STAR Foundation have been directly impacted by this awful pandemic. While STAR currently remains in a healthy financial position, we are facing uncertainty in our moderate-term and long-term forecasting like other charities and organizations around the globe. Therefore, in addition to considering a donation through one of the many options (listen herein), we ask that you consider making a non-binding pledge for our records.

We hope the pledge option affords our generous donors the following benefits:

- the ability to show support for those that are currently unable to make a financial commitment but believe they will be able to do so in the future;
- tax planning of charitable donations (e.g., timing of a contribution on January 1, 2021, for example);
- the ability to plan the various financial, charitable commitments within the year during challenging economic times;
- the convenience of being provided a simple reminder from STAR for ease of payment.

Finally, it will benefit STAR by improving our financial forecasting, our sponsorship of fundraisers, all of which are dedicated to directing as much money to research as possible and driving results for all Salla stakeholders.

On behalf of the entire Board, thank you for your tremendous generosity. We have been humbled by the support we have received; furthermore, we, as a STAR foundation, entirely run by volunteers, are inspired to use these gifts with a highest and best use to fund a future where no family faces Salla alone. Thank you again.

Make a pledge at SallaResearch.org/Pledge

Support STAR at SallaResearch.org/Support
As STAR was being represented in the Dominican Republic and Sweden, my husband Michael and I were sharing our experiences with a new group of families at the Albert Einstein College of Medicine. Much like our initial “Think Tank” in September 2018, families from around the world met to discuss a path forward for a rare disease afflicting their children called KDM5C. STAR has far surpassed the expectations of the new initiative launched by Montefiore Children’s Hospital and the Einstein College of Medicine, called “Operation IDD Gene Team.” This program allows for families and doctors to connect in an easier way with hopes to promote science and awareness into the rare disease that plagues their children. We had the honor of mentoring the families with KDM5C in starting their own foundation, building relationships with doctors, fundraising ideas, and the needed government paperwork to become a foundation, among other issues.

Knowing that our success in STAR is what budding foundations strive for is so inspiring! We are impacting other rare disease families in a significant way and this is truly because of the strong relationships all of the Salla families and supporters have built together.

To our Salla families, friends, and supporters, please read this newsletter with a renewed sense of hope. Rest assured that we will remain steadfast amidst the chaos and uncertainty of COVID-19. We are truly pioneering the outcome and science of this disease.

With warmest regards and deepest thanks,

Jessica

“It’s quite a scary path to walk on, when you have no one’s past experience to lean on, or learn from.

It’s all new and uncharted... but nothing is finished unless it is started.”