



ARYRE

ASXL RARE RESEARCH ENDOWMENT

Donor Summary

2018



FOREWORD

Dear Friends

On April 6, 2018, the AARE foundation was born to support research that will increase our understanding of the ASXL genes and to improve the treatment of individuals with congenital ASXL mutations.

Families have struggled with extraordinary challenges for decades due to the lack of evidence-based research and significant unknowns about ASXL syndromes.

These syndromes – Bohring-Opitz, Shashi-Pena & Bainbridge-Ropers – are under-diagnosed due to the challenges recognizing the diagnosis, shortage of genetic services, and the cost of genetic testing.

Through research AARE aims to improve the longevity and quality of life for these affected individuals and their families.

AARE key tenets include good stewardship of our resources and transparency.



Our donor summary includes the core focus areas where donations benefit ASXL syndrome patients.

Thank you for making a difference in the lives of these families.

Laura Badmaev





“ The amount of achievements this organization has had in less than a year since its beginning is nothing short of outstanding! I am so proud to be part of its journey! – *anonymous*



PRIORITIES

ASXL Registry & Biobank - These are foundational tools based on patient data & samples, which researchers will utilize to study and increase our knowledge of these syndromes.

Research grants - Effective research will bring an understanding of the underlying mechanisms and potential treatments for ASXL syndromes.



Conferences - Bring together professionals and families to deepen our understanding and exchange ideas that will move our mission forward.

ASXL CoE - Establish hub hospitals around the world where many ASXL patients will see specialists who will build their clinical expertise and improve care plans.



“ AARE does so much to help people affected by the ultra-rare ASXL syndromes! They help families connect, help caregivers be better advocates and help bring much-needed awareness to medical professionals.” – *Colleen Racciopi*



ADVOCACY & COMMUNITY

31 ASXL Symposium
Attendees

338 Social Media
Followers

104 Newsletter
Subscribers



ADVOCACY & COMMUNITY

8 Research Spotlight
Symposium Series

2 Campaign
Fundraisers

6 Family Stories

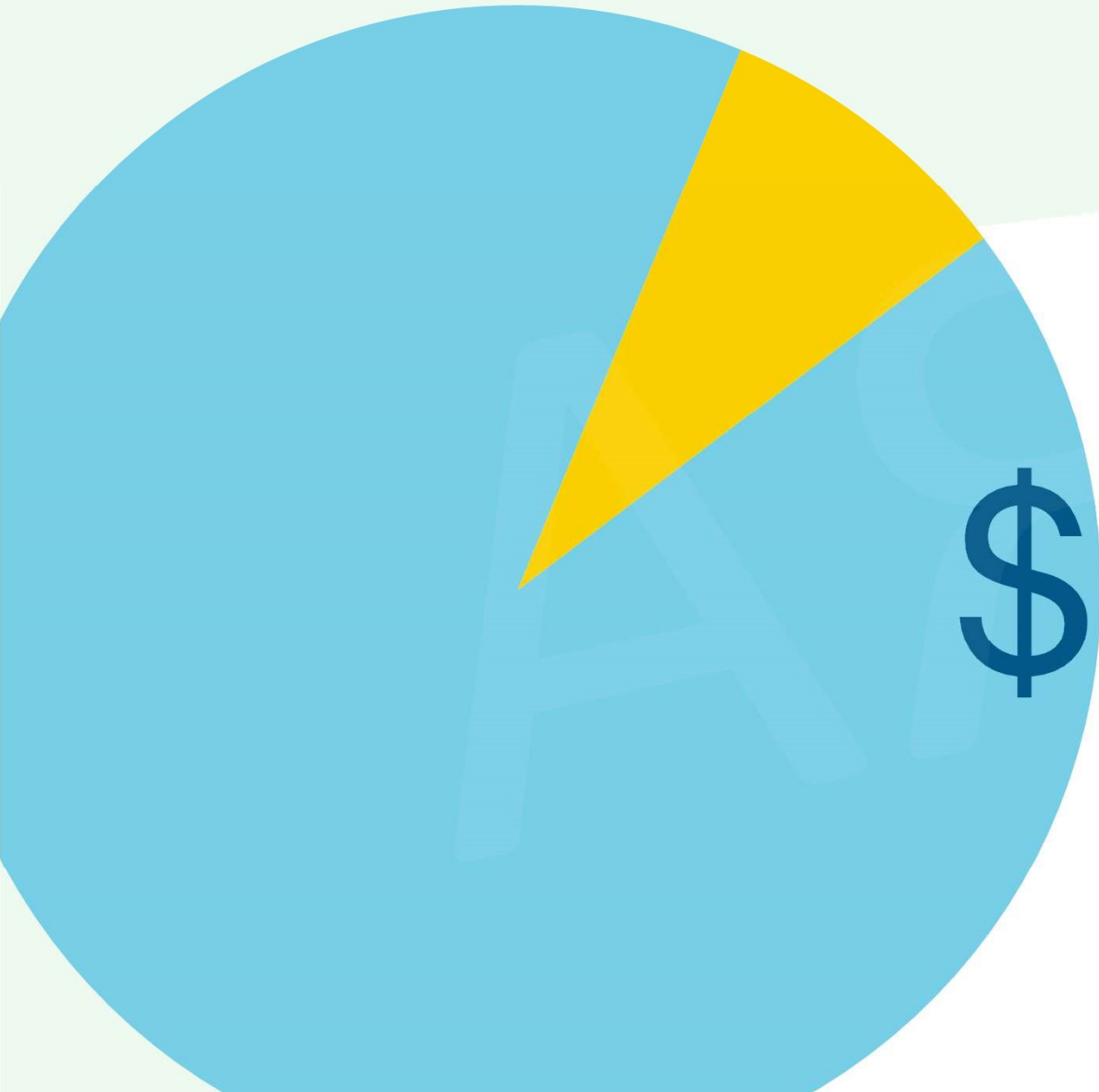
19 Education/
Health/Tips



“ The AARE Foundation means so much to our family. When my granddaughter was born, no one knew anything about Bohring-Opitz Syndrome. The AARE Foundation has given us hope! It funds true research to improve the lives of our children. – *Vonnie Iverson S.*



DONATIONS



Number of public donations 64

Average public donation amount \$59.32

44,796.21

- Public donations: 8.47 %
- Private donations: 91.53 %



“ We went to the conference sponsored by AARE and it was wonderful. For the first time we got to hear doctors that are studying these rare diseases and connect with other parents and families. They are doing a great job! – *Julie Chandler L.*

EXPENSES

\$ 31,272.85



Marketing & Fundraising Expenses (website and credit card donation fees)	934
Scientific Conference (UCLA, July 2018)*	11,060
Biobanking	10,944
Professional Services (lawyer, accountant, incorporation & license fees)**	8,025
General Expenses	308

COST PER \$ RAISED \$0.09



* UCLA subsidized the cost of the conference and contributed an additional \$13,413 toward the expenses.
** One-time expenses for the set up of the Foundation and required for private donation eligibility



“ We are thankful for non-profits like ASXL, we hope that with continued research we will know more about how we can help our little ones! Rare diseases, like Shashi-Pena Syndrome-ASXL2 need LOTS of TLC! And of course "ALL" rare disease need TLC! – *Michele P.*



RESEARCH STRATEGY

Effective
Research

Quality of
Life &
Cure

ARRR will develop a diverse research portfolio that will build on successful studies as well as support innovative investigations with high potential rewards. Key focus areas include:

- Gain or loss of function
- Transcripts expressed post natally
- Protein expression
- Pathogenic variants



LOOKING AHEAD

Reach for a Cure

The acceleration of technology provides optimism for our future. Whole Exome Sequencing and genetic testing are becoming more available and more families are receiving a diagnosis at an earlier age.

Precision medicine models existing drugs to determine which are effective and optimize them for rare diseases.

Patient cell lines can be converted to organoids — brain, stomach, blood, and lung tissues — that can express the same defects as those seen in patients and can be leveraged for trial therapeutics.



ARRR will strategically invest in our core priorities to bring our vision to fruition. We look forward to a successful 2019 and invite you to join us on this incredible journey!

Laura Badmaev



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Thank you!



“ The AARE Foundation is so important for families with children with an ASXL mutation, because they bring researchers and so knowledge together which is very important to understand more about these syndromes! The first conference in July 2018 was a very big success and brought families from all over the world together.” – *Suzanne Driessen*



LEAVE YOUR LEGACY

Planned giving is an opportunity to make a significant difference in the lives of these children and their families.

To learn more and discuss planning your gift to ARRE Foundation, please contact info@arrefoundation.org.

- Securities
- Real Estate
- Gifts by Will
- Life Insurance Policy
- Charitable Gift Annuity
- Charitable Remainder and Lead Trusts
- IRA Distributions



ARRE needs assistance from family, friends, caregivers, and neighbors to make our vision possible. We are a volunteer based organization and your support is critical.

VOLUNTEER

Sign up to receive news and updates about ASXL Rare Research Endowment activities.

SUBSCRIBE

You can help the ASXL Rare Research Endowment remain at the forefront ASXL research: arrefoundation.org/donate

DONATE

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ASXL RARE RESEARCH ENDOWMENT

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