

Patient-advocacy funded research in SCN2A-related disorders: A return on investment for FamilieSCN2A Foundation



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INTRODUCTION:

Patient advocacy groups have a growing importance in the strategic decisions that help accelerate research in the rare epilepsies, particularly with the FDA's increased emphasis on patient-focused drug development, clinical outcomes assessments (COAs) and patient-centered outcomes. The FDA has issued guidance that promotes the inclusion of patients, caregivers, and patient advocacy organizations. Thus, the importance of thoughtful capital deployment by advocacy organizations in the epilepsies is paramount.

BACKGROUND:

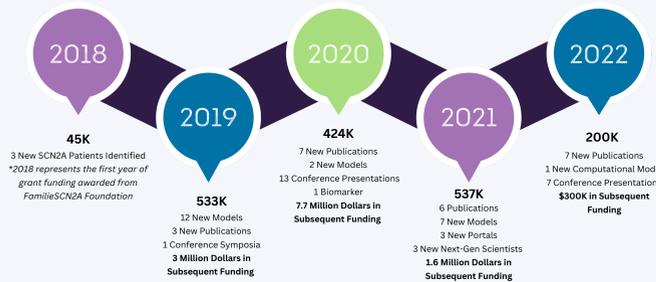
Since 2018, The FamilieSCN2A Foundation has awarded ~\$4.5 million dollars in grants supporting 22 projects with the purpose of advancing and accelerating SCN2A-related disorder (SRD) research. This funding has established a foundation of knowledge and resources that have become self-perpetuating and are rapidly propelling The FamilieSCN2A Foundation towards its vision of a world with effective treatments and cures for all SRDs.

METHODS:

A grant tracking system, curated by The FamilieSCN2A Foundation's scientific director, measured the return on investment of each grant awarded by the organization since 2018. Deliverables over time have been grouped into four categories: 1) number of corresponding publications, 2) number of models developed, 3) number of conference presentations / symposia / posters, and 4) amount of subsequent funding.

RESULTS:

FIVE-YEAR RETURN ON INVESTMENT



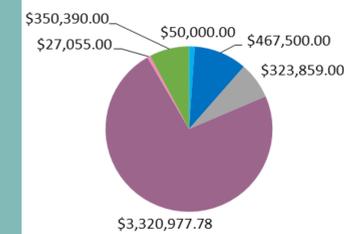
THESE AWARDS HAVE LED TO:



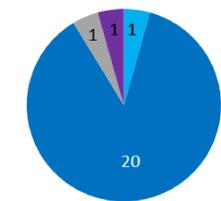
SINCE 2018

Types of Investments	Capital Deployed	Subsequent Funding	Publications	
			Models	(Peer Reviewed)
Basic Science	\$ 50,000	\$ -	0	1
Young Investigator	\$ 467,500	\$ 12,064,000	5	20
Natural History/Endpoint Assessment	\$ 323,859	\$ -	0	1
Clinical	\$ 27,055	\$ -	0	0
Drug Discovery/Development	\$ 3,320,978	\$ 500,000	20	1
Biomarker Discovery	\$ 350,390	\$ -	0	0
	\$ 4,539,782	\$ 12,564,000		
				2.8X ROI

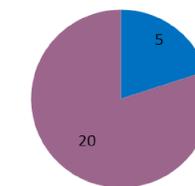
CAPITAL DEPLOYMENT



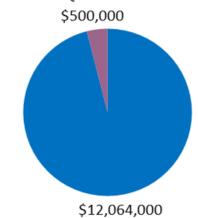
ROI: PEER REVIEWED PUBS.



ROI: MODELS CREATED



ROI: SUBSEQUENT FUNDING



KEY:
■ YOUNG INVESTIGATOR
■ DRUG DISCOVERY
■ NATURAL HISTORY / ENDPOINT SELECTION
■ BIOMARKER DISCOVERY
■ BASIC SCIENCE
■ CLINICAL

CONCLUSION:

Patient advocacy groups are instrumental in advancing research in the epilepsies and their return on investment. Given the increased interest by the FDA and other stakeholders to include patient advocacy organizations in the drug development process, it is imperative that patient advocacy organizations consider the impact of their dollars and how these investments may generate further research in their respective diseases.