12:15 – 1:15 PM	1 hr	NETWORKING	Encouraged opportunity to meet and network with speakers and fellow participants	
1:15 – 2:00 PM	45 min	Mike Graglia, SynGAP Research Fund, Inc.	Early Research Funding: Parent Experience	Mike Graglia, Founder of the SynGAP Research Fund will share his personal experiences in building a disease foundation from scratch and funding strategies.
2:00 – 2:30 PM	30 min	Charlene Son Rigby, Global Genes	Navigating Foundation Dynamics	Charlene Son Rigby, CEO of Global Genes will discuss the nuances of navigating the complexities around building and managing relationships as a patient foundation.
2:30 – 3:30 PM	1 hr	Tom Kassberg, Ultragenyx Beryl Cummings, Third Rock Ventures Neil Kumar, BridgeBio	Panel Discussion: Late- stage Funding – Partnerships and VCs	Tom Kassberg, CBO of Ultragenyx, Beryl Cummings, Senior Associate at Third Rock Ventures and Neil Kumar, CEO of BridgeBio will lead a panel discussion on partnering and funding.
3:30 – 3:45PM	15 min		BREAK	
3:45 – 4:15 PM	30 min	Casey McPherson, To Cure a Rose Foundation	Why a parent makes the perfect founder	Parent Journey
4:15 – 4:45 PM	30 min	Julia Vitarello, Mila's Miracle Foundation	From Mila to Millions	Parent Journey

WHATEVER YOU CAN DO, OR DREAM YOU CAN, BEGIN IT. **BOLDNESS HAS GENIUS, POWER** AND MAGIC IN IT. [GOETHE]



SYNGAP RESEARCH FUND

Collaboration. Transparency. Urgency.

Mike - Tony's Dad & SRF's Founder

Left my career a few years ago to lead SRF. In addition to working with the team of SynGAP families and partners, I serve on Executive Board of COMBINEDBrain, AES Epilepsy Research Benchmarks Stewards Committee and work closely with Innovation and Value Initiative Methods Summit & Personalized Medicine Coalition and FasterCures Leaders Link.











Professional background in global development, healthcare strategy, finance and planning at top-tier institutions.









Educational background in Mathematics (BS), International Economics (MA) and Finance (MBA).

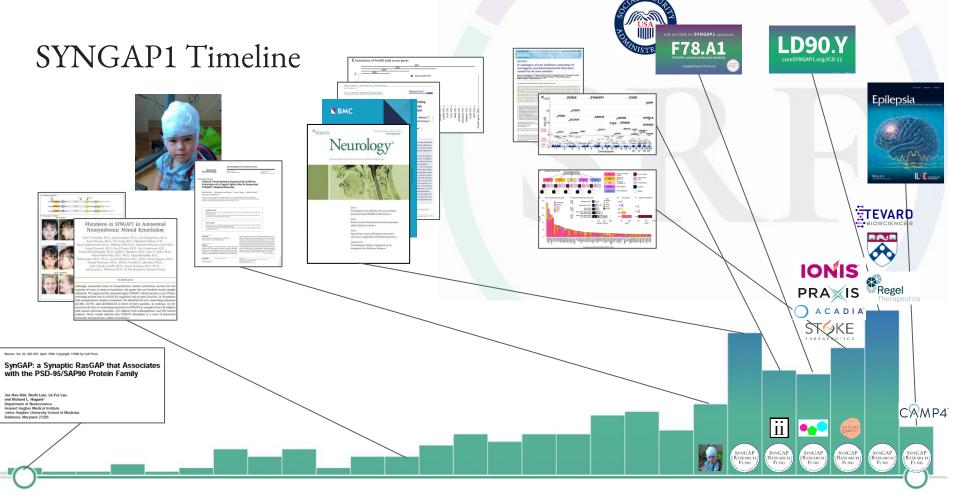




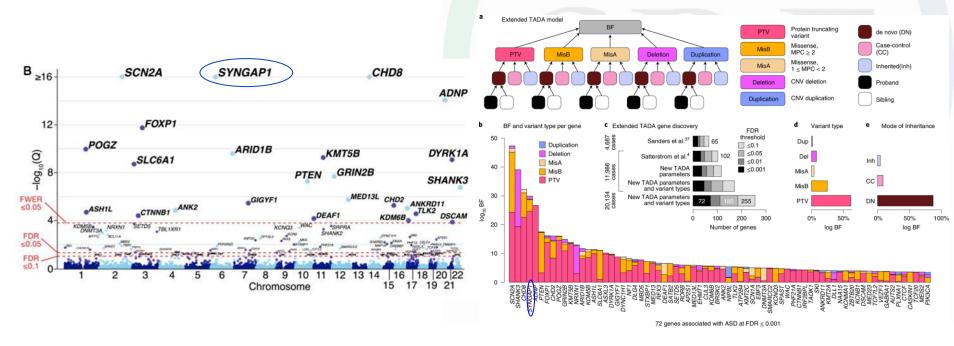




If content is King, then context is God.

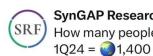


SYNGAP1 highly associated with Autism



Satterstrom et al. Large-Scale Exome Sequencing Study Implicates Both Developmental and Functional Changes in the Neurobiology of Autism. *Cell.* **2020** Feb 6;180(3):568-584.e23.

Fu JM, Satterstrom FK, Peng M, et al. Rare coding variation provides insight into the genetic architecture and phenotypic context of autism. *Nat Genet*. **2022**;54(9):1320-1331.



SynGAP Research Fund (SRF) @cur... · 5h ···

How many people have SYNGAP1?

The #SyngapCensus

Syngap.Fund/Census



=114

99

■95 **=**50

148

≤ 47 **≥** 40

38

28

21

14

12

1110

9

1 2 3 8

2 2 3 3

+6

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3 5 6 6

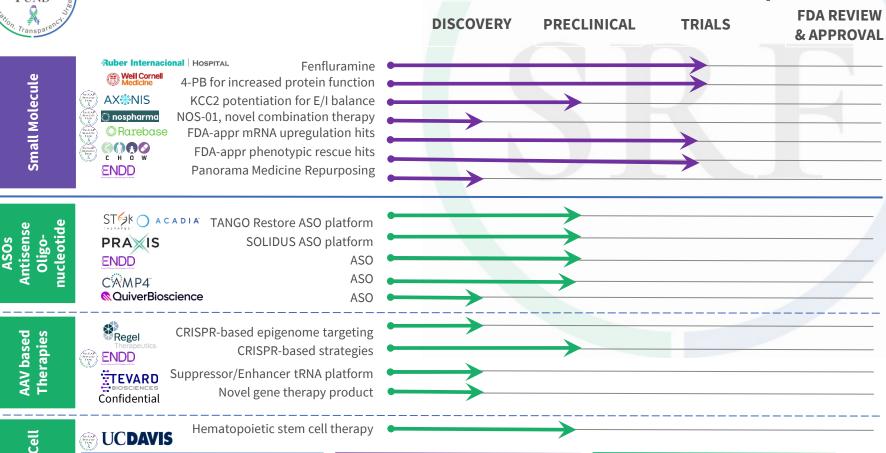
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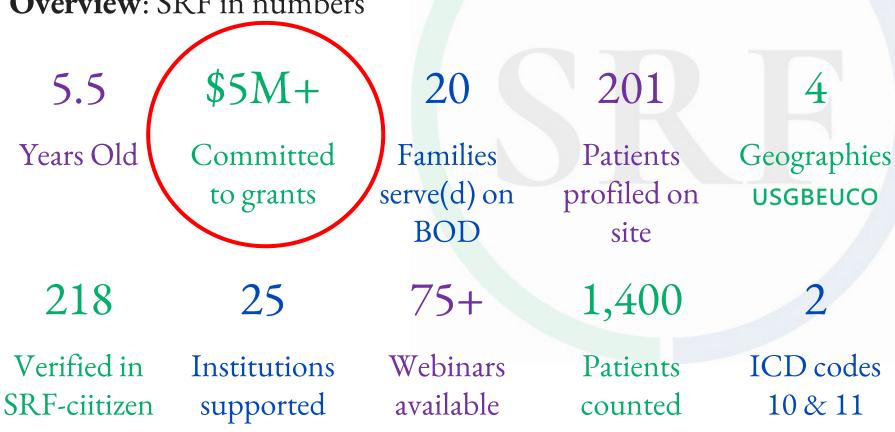


SYNGAP1 THERAPEUTIC PIPELINE | 2024





Overview: SRF in numbers



Current Grants - Committed

y	Project	Institution	SRF Funding	2019	2020	2021	2022	2023	2024
	Animal Models	Hopkins	\$250,000			C.			
	Accelerate Current Research	Baylor	\$130,000						
Models	Patient Derived Organoids	USC	\$306,500						
Models	Patient Derived Cell Lines	Rarebase & CB	\$63,750						
	Animal Models	UBC	\$20,000						
	Two mouse models	Jackson Labs	\$42,700						
	Accelerate Current Research	Scripps	\$205,000						
	Function Drug Discovery	Rarebase	\$577,500						
	Confidential - Pre-clinical work	McGill	\$120,000						
Small Molecules / Drug Repurposing	E/I Imbalance small molecules	Harvard	\$140,000						
Drug Kepurposing	4PB Academic Trial*	Cornell	\$270,000						
	Fly Screen	Utah	\$65,500						
	Myelin Plasticity	Stanford	\$130,000						
	ASO Development	Hopkins	\$250,000					Γ	
	Epigenetic Tx Opportunities	UPenn	\$165,000						
	Novel Delivery: Exosome	UNL	\$99,909						
	Hematopoietic stem cells	UC Davis	\$130,000						
Gene Therapies	Targetable defects in missense	Turku	\$180,000						
	tRNA Tx POC	Hopkins	\$130,000						
	AAV POC in Rats	Edinburgh	\$180,000						
	Missense portal & structure	Turku	\$100,000						
	Ciitizen / Rare Patient Network	Invitae	\$80,000						
Patient Data	Develop NHS with Ciitizen	BCH	\$237,000						
Patient Data	Launch PRO Platform	RareX	\$0						
	SYNGAP1 in older patients	Toronto	\$156,500						
	DSC EEG Study	ВСН	\$77,000						
D: 1 /	Validated Scales: NET	John Carroll	\$15,000						
Biomarkers / Endpoints	Validated Scales: ORCA	CB/Duke	\$0						
Lindpoints	Undiagnosed Screening Tool	Probably Genetic	\$15,600						
	Scales: NET phase 2	John Carroll	\$100,500						
* Grant approved, agreen	nent with lawyers, not yet announced		\$4,237,459	*Through first	half 2023, \$772k	committed in 21	nd grant cycle 20	23 (total now >\$5M)

It gots complicated fact. I have a 20 grants in flight to day

= Remaining +

\$213,750

\$65,000

\$183,545

\$41.043

Fully Funded

Fully Funded

Fully Funded

Fully Funded

\$65,000

\$13,609

\$50,000

\$78,190

\$15,000

\$100,000

\$50,000

\$7,000

\$186,700

\$196,672

\$130,000

\$128,888

\$130,000

\$186,700

\$2,000,265

\$65,377

\$10,000

\$130,000

\$105,000

\$100,000

\$156,380

\$25,000

\$100,000

\$50,000

\$7,000

\$186,700

\$196,672

\$130,000

\$128,888

\$130,000

\$186,700

\$5,290,252

11	It gets complicated fast - I have ~20 grants in flight today.											
Year	Institution	Prof/Focus	Commitment	= Remaining +	Year	Institution	Prof/Focus	Commitment				
2018	Hopkins	Huganir	\$500,000	Fully Funded	2022	Rarebase	FM 38, 40, 41, 45	\$570,000				
2018	Scripps	Rumbaugh	\$205,500	Fully Funded	2022	Hopkins	Coller	\$130,000				
2018	Baylor	Holder	\$130,000	Fully Funded	2022	Edinburgh	Cobb/Kind	\$183,545				
2019	UCSF	Lowenstein	\$10,000	Fully Funded	2022	USC	Quadrato	\$130,000				
2019	Penn	Heller	\$130,000	Fully Funded	2022	Harvard/BCH/Axo	r Xin/Kadam	\$140,000				
2020	Ciitizen	Brimble	\$40,000	Fully Funded	2023	Cornell	Cunnane (Med Stu)	\$5,000				
_0_0	Onti-On	51111510	Ψ.0,000	i dily i dilaba								

Fully Funded

Fully Funded

Fully Funded

Fully Funded

\$65,133

Fully Funded

Fully Funded

Fully Funded

Fully Funded

Fully Funded

Fully Funded

\$20,000

Fully Funded

Fully Funded

\$15,647

\$0

\$58,388

\$46,500

\$14,200

\$130,000

\$70,191

\$238,133

\$63,750

\$1,286

\$150,000

\$19,672

\$99,909

\$15,600

\$148,000

\$1,250

\$65,000

\$42,700

\$187,299

\$115,000

2020

2020

2020

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2022

USC

JCU

USC

Harvard

Harvard

Rarebase

Rarebase

Nebraska

UC Davis

Penn

Turku

McGill

Combined Brain

Canada Models

Probably Genetic

Fundacion NICE

Jackson Labs

Coba / Quadrato

Function, round 1

UBC & Montreal

Zempleni

Aledo

MDBR

Courtney

TBD

Bowie

Lukas Lange

Joe Anderson

Frazier

Coba

DSC

Poduri

IPSC

IPSC

2023

2024

2023

2023

2023

2023

2023

2023

2023

2023

2023

2023

2024

2024

2024

2024

Utah

JCU

Turku

Toronto

Ottawa

Cornell

NYU

Jax

Florey

UCSF

Jax

Nebraska

U. Ottawa

Edinburgh (UK)

AES/CHOP

Stanford

Chow

McKee

Knowles

Frazier

Postilla

Andrade

Lacoste

Grinspan

Devinsky

Zempleni

Willsey

Lacoste

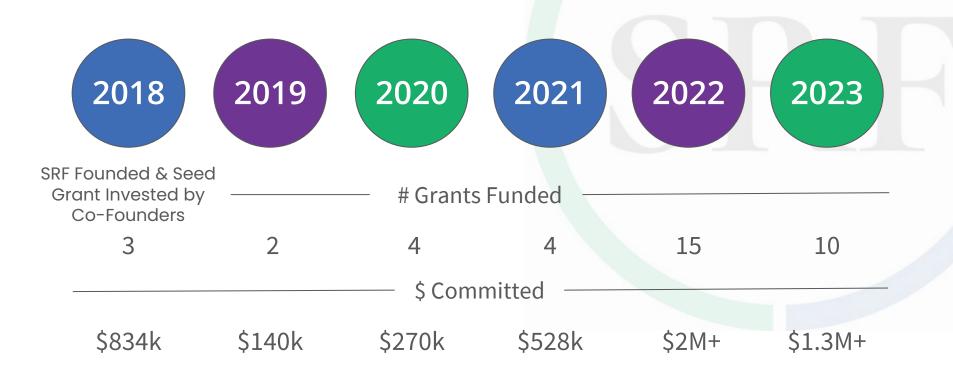
Simon

Donlin-Asp

Waters

Simon

Financials



•1 1 1 1

Dana com	$\frac{u}{u}$	<u>iid</u> ievei	ase sivi	118. 1.2.				
		2018	2019	2020	2021	2022	Total	Notes
990 Revenue (line 12)	А	\$20,000	\$382,888	\$590,087	\$1,357,074	\$2,144,228	\$4,494,277	
990 Expense (line 18)	В	0	\$52,910	\$225,407	\$874,679	\$1,192,705	\$2,345,701	
Net	A - B	\$20,000	\$329,978	\$364,680	\$482,395	\$951,523	\$2,148,576	
Direct Payments	F = C+D+E	\$361,833	\$0	\$0	\$250,000	\$0	\$611,833	Donors direct
Total SRF Raised	G = A+F	\$381,833	\$382,888	\$590,087	\$1,607,074	\$2,144,228	\$5,106,110	
Total SRF Spent	H = B+F	\$361,833	\$52,910	\$225,407	\$1,124,679	\$1,192,705	\$2,957,534	

13.8%

\$51,701

\$51,701

13.50%

\$255,000

\$306,701

80.10%

\$0

\$0

0

38.2%

\$51,758

\$51,758

8.77%

\$84,500

\$136,258

23.09%

\$0

\$0

dia comi	indifficy <u>e</u>	<u>ura</u> rever	age givi	115. 1.2.	9
		2018	2019	2020	
Revenue (line 12)	Α	\$20,000	\$382,888	\$590,087	

94.8%

\$20,000

\$111,833

\$131,833

\$250,000

\$381,833

100.00%

34.53%

			****	^	
		2018	2019	2020	
bulla comn	nunity <u>a</u>	<u>ina</u> levei	rage givi	ng: 1:2:0	5

Build community	and leve	rage givi	ng: 1:2:	(
	2018	2019	2020	Ī

H/G

С

K = I + J + C

K/G

M = D + E

N = K + L + M

N/G

Spend

Founders Gifts to SRF

Founders Gifts via other

Founders Direct giving

Total Founder Spend

Founders Soft Credits

Founder Direct Donors

Total Founder Impact

% SRF Revenue

% SRF Revenue

1:2:	6		
020	2021	2022	Tota

70.0%

\$0

\$0

\$209,317

\$209,317

13.02%

\$51,079

\$250,000

\$510,396

31.76%

55.6%

\$0

\$175,000

\$175,000

\$233,006

\$603,006

195000

28.12%

8.16%

57.9%

0 Need to find

62.

\$507,776

\$111,833

\$619,610

\$695,000

37.96%

\$1,938,195

12.13%

\$623,585 Conservative

2023 Event Recap

- Million Dollar Bike Ride | \$31k raised + \$30k match
- Sprint4Syngap | \$252k raised
 - Tavillas carry this event (\$166k raised)
- 3rd Annual Cannonball for a Cure | \$130k raised
- 3rd Annual SRF Gala (Caren Leib | \$80k raised
- 2nd Annual Scramble for Syngap | \$25k raised
- 2nd Syngap Soiree | \$300k+ raised
- 1st Annual Syngap Paddleslap | \$20k raised



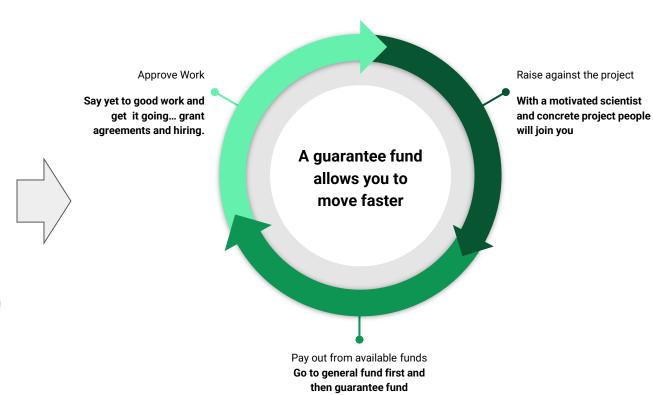


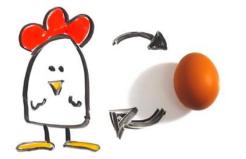




A guarantee fund can allow you to move faster

We can't raise funds till we have a project and we can't start a project till we have funds...





What I think is important after working on this for 5+ years

Three Essential Concepts - These are ideas that you have to embrace if you are seriously going to do some fundraising

Four Major Learnings - Realizations I've had along the way and want you to know.

Ten helpful tips - These are things I've learned, generally by doing the opposite first. I have iterated relentlessly and made so many mistakes.

Schedule A (Form 990) 2022

SynGAP Research Fund Incorporated

83-1200789

Page 2

Part II Support Schedule for Organizations Described in Sections 170(b)(1)(A)(iv) and 170(b)(1)(A)(vi)

(Complete only if you checked the box on line 5, 7, or 8 of Part I or if the organization failed to qualify under Part III. If the organization fails to qualify under the tests listed below, please complete Part III.)

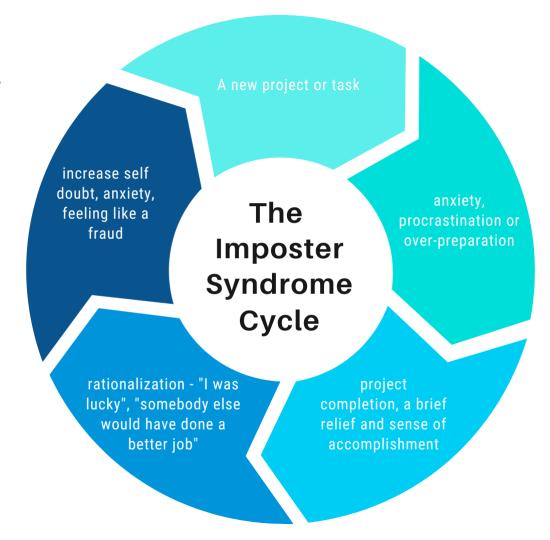
Section A. Public Support

Calendar year (or fiscal year beginning in)	(a) 2018	(b) 2019	(c) 2020	(d) 2021	(e) 2022	(f) Total
Gifts, grants, contributions, and membership fees received. (Do not include any "unusual grants.")	20,000.	382,888.	590,087.	1,364,391.	2,144,228.	4,501,594.

You are exactly where you need to be.

Don't waste time doubting yourself, your loved ones can't afford it.

Jump in and own what you are doing, it's incredibly hard and important. You can't afford self-sabotage.



The universe is naturally abundant

You have to have this mindset if you are fundraising.

Do not talk yourself out of believing you will find the money.

If you don't believe it, who will?



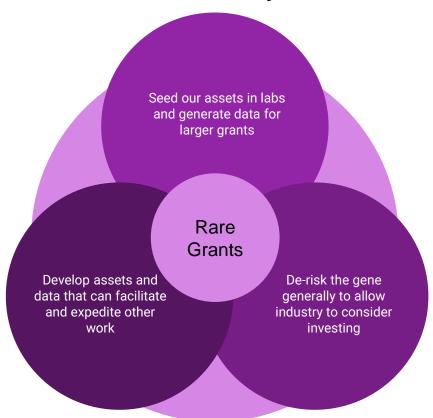
Our capital is rare - Seed, Risk tolerant & Catalytic

What do you say when they ask "Why should I give you money?"

Our deep commitment to disease X means we will take risks nobody else will, this isn't an opportunity, it's an obligation.

It can be catalytic to other capital — otherwise we are kidding ourselves — we are also scaffolding or derisking the target.

- 1. Getting researchers data for larger grants and seeing out assets in their labs.
- 2. Scaffolding the target Developing assets like iPSCs or models.
- 3. De-risking the target Developing clinical trial endpoints, natural history, etc.



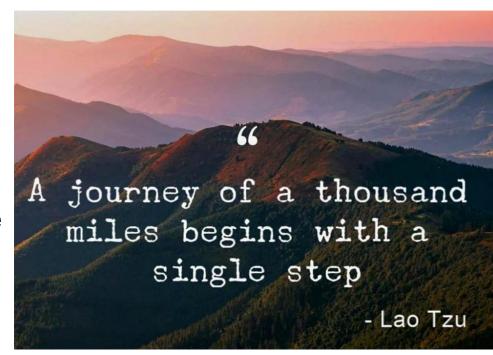
e.g. Your work increases your scores on tables like this.

	US / EU	Target	Gene				Tx	Nat.	PC	Clin.	Pat.				Clin.	Mkt.	
Disease Name	Preva lence	Gene	Size	Inher.		Onset	Me ch.	Hist.	Mod.	Dev.	Org.	SZ	ID	MD	Need	Орр.	Disease Summary
	Internal Development Candidates - Genetic Neurodevelopmental Disorders & Epileptic Syndromes																
SCN2A-Related Disorders	62,133	SCN2A	6,018	AD		<12 m	4	5	4	4	5	4	5	3	5	5	Moderate-severe ID, autism; refractory epilepsy
SCN8A-Related Disorders	57,275	SCN8A	5,943	AD		<6 m	3	5	4	3	4	3	5	4	4	5	Severe ID, refractory epilepsy with regression
Dravet Syndrome	56,031	SCN1A	5,997	AD		<12 m	5	5	5	5	5	5	5	4	5	3	Severe-profound ID, DD & intractable epilepsy
CHD2 Encephalopathy	53,183	CHD2	5,487	AD		<2 y	5	5	4	4	1	5	4	2	4	5	Moderate-severe ID with refractory epilepsy
Phelan-McDermid Syndrome	51,321	SHANK3	5,193	AD		<2 y	5	5	4	4	5	4	5	4	5	5	Moderate-severe ID, DD autism; seizures in 40%
SYNGAP1 Encephalopathy	47,491	SYNGAP1		AD		<2 y	5	5	5	5	5	5	5	4	5	5	Severe ID with up to ~200 seizures per day
GRIN2B-Related Disorder	45,960	GRIN2B	4,455	AD		<4 y	4	4	1	3	5	4	5	4	4	4	Moderate-severe ID; 50% epilepsy, >25% autism
1A Neurological Disorder	40,559	KIF1A	5,073	AD		<2 y	4	4	3	4	5	3	4	4	5	5	Severe ID, epilepsy; regression
Rett Syndrome	38,879	MECP2	1,461	XL		<18 m	4	5	5	4	5	4	5	5	5	4	Neurodegeneration with ID, autism & early death
Smith-Magenis Syndrome	31,103	RAI1	5,721	AD		<6 m	5	4	5	4	5	2	5	3	4	5	Moderate ID, DD with autism; no epilepsy
DNM1 Encephalopathy	29,408	DNM1	2,595	AD		~6 m	4	5	5	5	5	5	5	5	5	5	Profound ID, DD with intractable epilepsy
STXBP1 Encephalopathy	28,108	STXBP1	1,785	AD		<8 m	5	5	4	5	5	5	5	4	5	5	Profound ID, DD with resistant epilepsy & autism
KCNQ2 Encephalopathy	25,487	KCNQ2	2,619	AD		<1 m	4	4	4	4	5	5	5	5	5	5	Severe ID, DD and neonatal onset epilepsy
SLO8A 1-Related Disorder	20,603	SLO8A1	1,800	AD		<4 y	5	4	2	4	5	4	4	3	4	5	Moderate ID, epilepsy with regression
CDKL5 Deficiency Disorder	16,888	CDKL5	2,883	XL		<3 m	4	5	4	5	5	5	5	. 5	5	3	Devastating DD, ID & epilepsy
GABRB3 Associated Epilepsy	16,633	GABRB3	5,783	AD		<12 m	4	5	4	4	3	4	4	3	4	5	Moderate ID, resistant epilepsy; ~25% autism
SETD5 Syndrome	12,388	SETD5	4,329	AD		<2 y	5	4	5	3	2	1	4	2	4	5	Moderate ID & DD; ~25% autism
PRRT2 Dyskinesia & Epilepsy	11,664	PRRT2	1,023	AD		<1 y	4	4	4	3	1	4	3	4	3	4	Infantile epilepsy, dyskinesia; some ID
CACNA1A-Related Disorders	7,778	CACNA1A	7,527	AD		<10 y	4	3	3	4	5	4	2	4	3	4	Highly variable spectrum
GLUT1 Deficiency Syndrome	7,422	SLC2A1	1,479	AD		<1 y	5	5	5	4	5	5	4	5	4	4	Moderate ID, DD, epilepsy & motor disorder

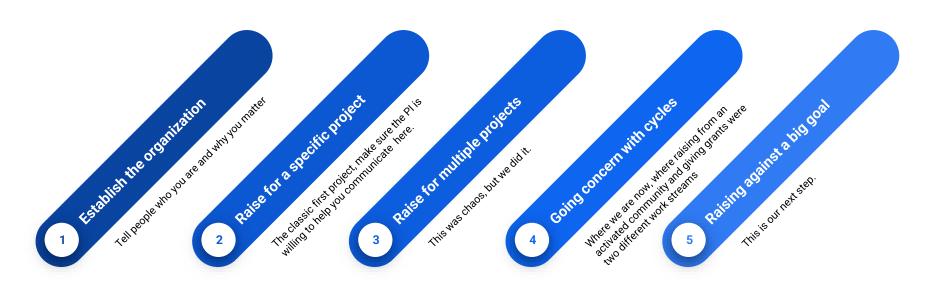
Language & culture matters: "We can fill an unmet need together" vs "I need to raise \$100k".

The sooner you go to the community with a project and urge them to support it, the sooner this gets real.

In addition to fundraising you are activating a community.



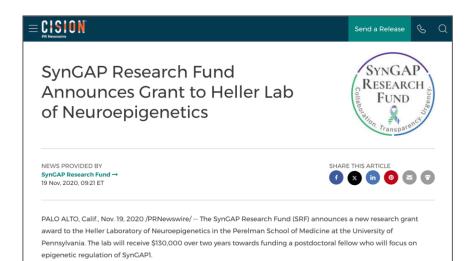
How you fundraise will change as you grow,



It's darkest before the dawn

A stalled fundraiser feels like a public failure, it's not.

These gaps are when people who can fill gaps step forward.



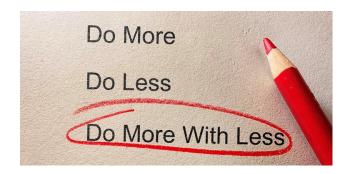


Donors don't love overhead, be ready to discuss.

Option 1: Good news we don't spend much on overhead!

You are just starting and working on a shoestring, so go ahead and own that.

Put a time limit on it.



Option 2: Refocus the conversation on capacity building.

We need a dedicated organization for this disease and organizations need funding to grow.

Help with your Ws: Wealth, Wisdom or Work.



10 Tips

- 1. Hire someone, this is a ton of work Virtual assistant or parent.
- 2. Plumbing matters, don't ignore it Scalable tech to move & track money.
- 3. Communicate relentlessly Podcast/Videos, Social, Newsletter.
- 4. Call people to thank and to ask You learn what matters in calls.
- 5. Throw and annual event ASAP Events trigger donations, get it going.
- 6. Don't pay overheads to large institutions They get it, put policy on your site.
- 7. Press Releases matter, use them. Eureka Alerts is reasonable.
- 8. Newly diagnosed families panic, give them something to do. Page for kiddo.
- 9. Don't skip the audit These are useful, credibility building and not that bad.
- 10. Liquidate stocks upon receipt You are not in the stock business.

SRF has three SYNGAP1 podcasts for patient finding & support



Episodes V

SATURDAY - VIDEO

113. Fondo sets the bar for LatAm Engage 2nd Soiree was a hit, grants are rolling in! Super Heroine: Vicky Arteaga Fondo Syngap is tirele https://www.syngapresearchfund.org/professionals/20

SvnGAP10 weekly 10 minute updates on SYN

Syngap Research Fund, 501(c)(3)

★ 5 (70) · NON-PROFIT · UPDATED WEEKLY



Episodes V

AUGUST 29

Mike Graglia, SRF Managing Dir. & SYNGAI difficult. Tony's experience shows that it's Show Notes Going back to school is difficult for most and other rare diseases, it is especially hard! Mike Grag



SYNGAP1 Stories

Syngap Research Fund, 501(c)(3)

PARENTING - UPDATED BIWEEKLY



Café SYNGAP1

Syngap Research Fund, 501(c)(3)

PARENTING - UPDATED BIWEEKLY

Bienvenidos al nuevo podcast de SRF en Español: Café Syngap1 pretender ser un espacio para encontrar apoyo, consejos y esperanza en una comunidad de Padres, Hermanos, Investigadores, Científicos, Terapeuta MORE

▶ Latest Episode













Episodes V

Episodio 01: Valeria Torcetta y su hija Charo- La Primera Paciente Diagnosticada con SynGAP1 en America Latina

Episodio #01 Bienvenidos a nuestro nuevo podcast, Café SynGAP1! En nuestro primer episodio, nos acompaña Valeria Torcetta, una invitada especial quien nos habla sobre Charo, su hija con Syngap1, y primera pa...







Science + L♥ve = Cure



Cure GABA-A in 5 | Episode 9









Thank you. mike @cure SYNGAP1.org @cureSYNGAP1