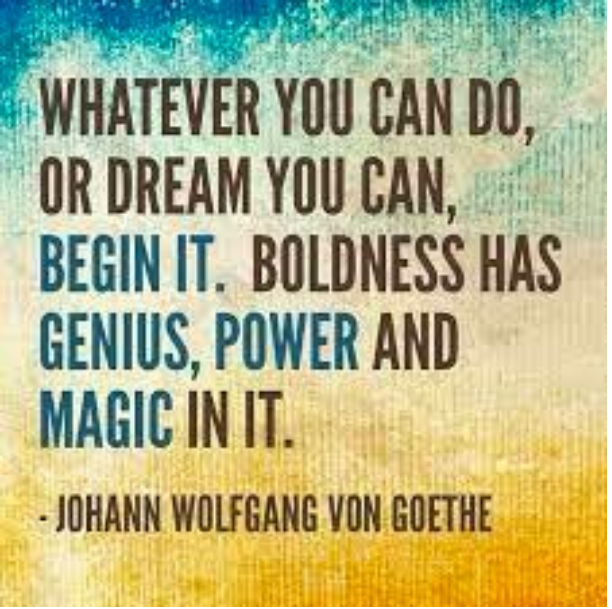


SynGAP Research Fund

Collaboration. Transparency. Urgency.

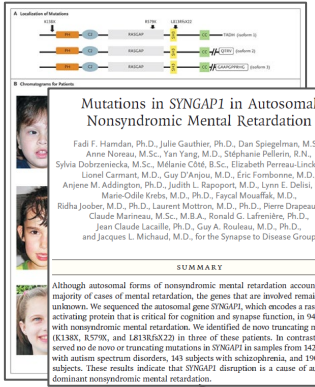
Mike - Tony's Dad & SRF's Founder

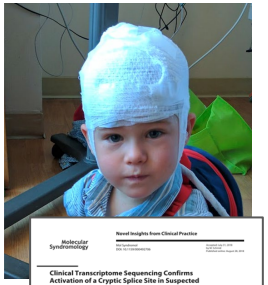
Left my career a few years ago to lead SRF. In addition to working with the team of SynGAP families and partners, I serve on Executive Board of COMBINEDBrain, AES Epilepsy Research Benchmarks Stewards Committee and work closely with Innovation and Value Initiative Methods Summit & Personalized Medicine

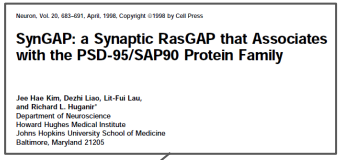
Professional background in global development, healthcare strategy, finance and planning at top-tier institutions

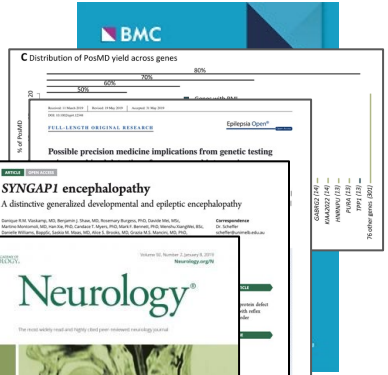
Educational background in Mathematics (BS), International Economics (MA) and Finance (MBA).

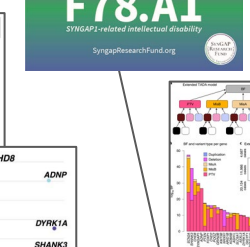
SYNGAP1 Timeline

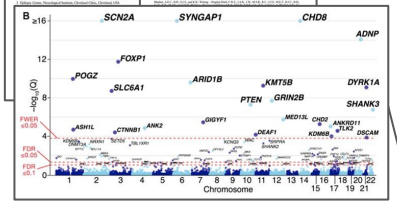


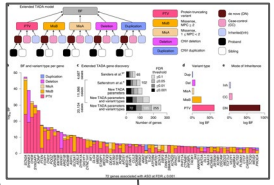












1998

247 PubMed.gov results for SYNGAP1 from 1998 to 2023, as of **Sept. 5, 2023**

2023

It gets complicated fast - I have ~20 grants in flight today.

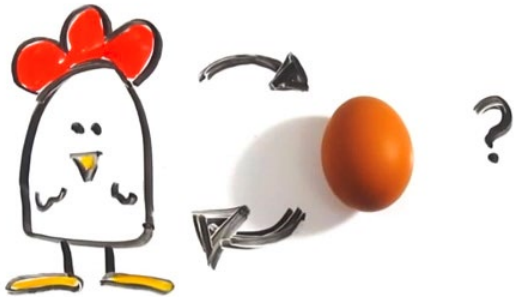
Year	Institution	Prof/Focus	Commitment	= Remaining +
2018	Hopkins	Huganir	$500,000	Fully Funded
2018	Scripps	Rumbaugh	$205,500	Fully Funded
2018	Baylor	Holder	$130,000	Fully Funded
2019	UCSF	Lowenstein	$10,000	Fully Funded
2019	Penn	Heller	$130,000	Fully Funded
2020	Ciitizen	Brimble	$40,000	Fully Funded
2020	USC	Coba / Quadrato	$46,500	Fully Funded
2020	JCU	Frazier	$14,200	Fully Funded
2020	USC	Coba	$130,000	Fully Funded
2021	Harvard	DSC	$70,191	Fully Funded
2021	Harvard	Poduri	$238,133	$65,133
2021	Rarebase	IPSC	$63,750	Fully Funded
2021	Combined Brain	IPSC	$1,286	Fully Funded
2021	Rarebase	Function, round 1	$150,000	Fully Funded
2022	Canada Models	UBC & Montreal	$19,672	Fully Funded
2022	Nebraska	Zempleni	$99,909	Fully Funded
2022	Probably Genetic	Lukas Lange	$15,600	Fully Funded
2022	UC Davis	Joe Anderson	$148,000	$20,000
2022	Fundacion NICE	Aledo	$1,250	Fully Funded
2022	Penn	MDBR	$65,000	Fully Funded
2022	Jackson Labs	TBD	$42,700	$15,647
2022	Turku	Courtney	$187,299	$0
2022	McGill	Bowie	$115,000	$58,388
2022	Rarebase	FM 38, 40, 41, 45	$570,000	$213,750
2022	Hopkins	Coller	$130,000	$65,000
2022	Edinburgh	Cobb/Kind	$183,545	$183,545
2022	USC	Quadrato	$130,000	$41,043
2022	Harvard/BCH/Axor	Xin/Kadam	$140,000	Fully Funded
2023	Cornell	Cunnane (Med Stu)	$5,000	Fully Funded
2023	Utah	Chow	$65,377	Fully Funded
2024	AES/CHOP	McKee	$10,000	Fully Funded
2023	Stanford	Knowles	$130,000	$65,000
2023	JCU	Frazier	$105,000	$13,609
2023	Turku	Postilla	$100,000	$50,000
2023	Toronto	Andrade	$156,380	$78,190
2023	Ottawa	Lacoste	$25,000	$15,000
2023	Cornell	Grinspan	$100,000	$100,000
2023	NYU	Devinsky	$50,000	$50,000
2023	Florey	Waters	$7,000	$7,000
2023	Jax	Simon	$186,700	$186,700
2023	Nebraska	Zempleni	$196,672	$196,672
2024	UCSF	Willsey	$130,000	$130,000
2024	U. Ottawa	Lacoste	$128,888	$128,888
2024	Edinburgh (UK)	Donlin-Asp	$130,000	$130,000
2024	Jax	Simon	$186,700	$186,700
			$5,290,252	**$2,000,265**

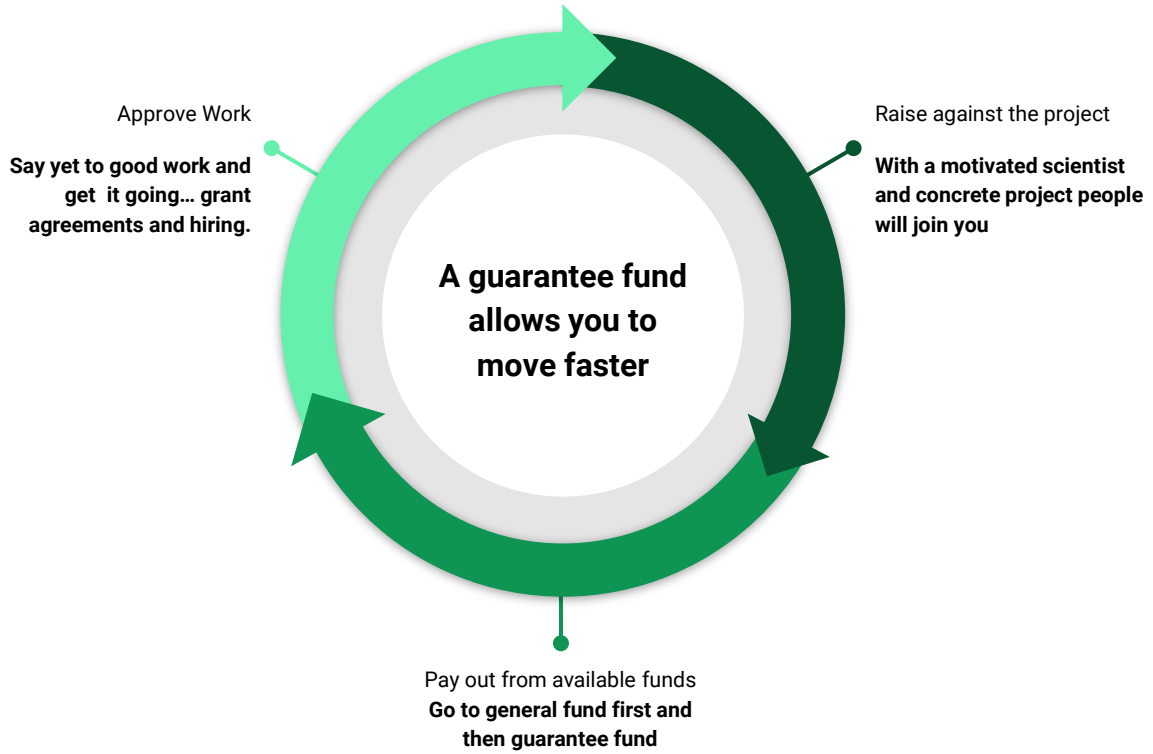
Build community and leverage giving: 1:2:6

		2018	2019	2020	2021	2022	Total	Notes
990 Revenue (line 12)	A	$20,000	$382,888	$590,087	$1,357,074	$2,144,228	$4,494,277	
990 Expense (line 18)	B	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	
Spend	H/G	*94.8%*	*13.8%*	*38.2%*	*70.0%*	*55.6%*	*57.9%*	
Founders Gifts to SRF	I	$20,000	$51,701	$51,758	$209,317	$175,000	$507,776	
Founders Gifts via other	J	0	$0	$0	$0	$0	0	Need to find t
Founders Direct giving	C	$111,833	$0	$0	$0	$0	$111,833	
Total Founder Spend	K = I + J + C	$131,833	$51,701	$51,758	$209,317	$175,000	$619,610	***62.***
% SRF Revenue	K/G	*34.53%*	*13.50%*	*8.77%*	*13.02%*	*8.16%*	***12.13%***	
Founders Soft Credits	L	0	$255,000	$84,500	$51,079	$233,006	$623,585	Conservative
Founder Direct Donors	M = D+E	$250,000	0	0	$250,000	195000	$695,000	
Total Founder Impact	N = K + L + M	$381,833	$306,701	$136,258	$510,396	$603,006	$1,938,195	
% SRF Revenue	N/G	*100.00%*	*80.10%*	*23.09%*	*31.76%*	*28.12%*	***37.96%***	

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.)

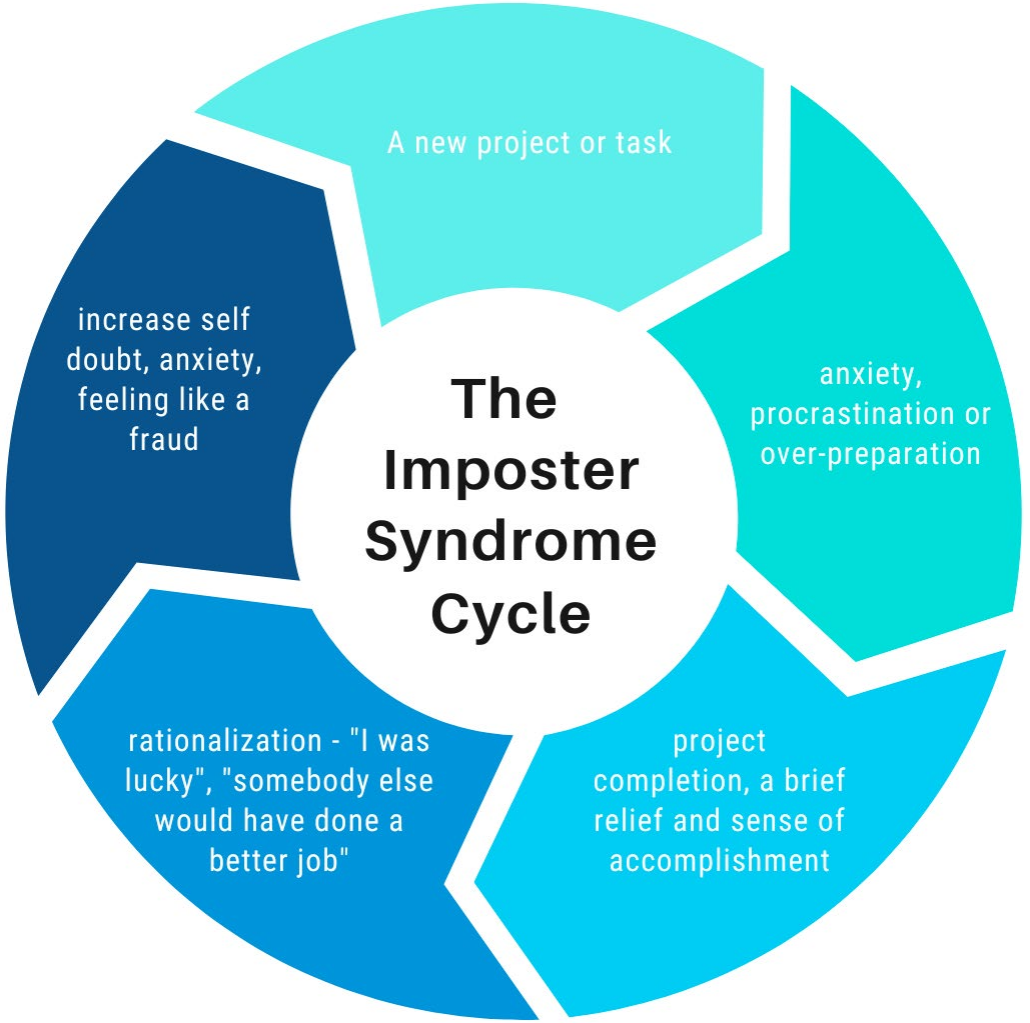
Section A. Public Support

Calendar year (or fiscal year beginning in)	**(a)** 2018	**(b)** 2019	**(c)** 2020	**(d)** 2021	**(e)** 2022	**(f)** Total
1 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?

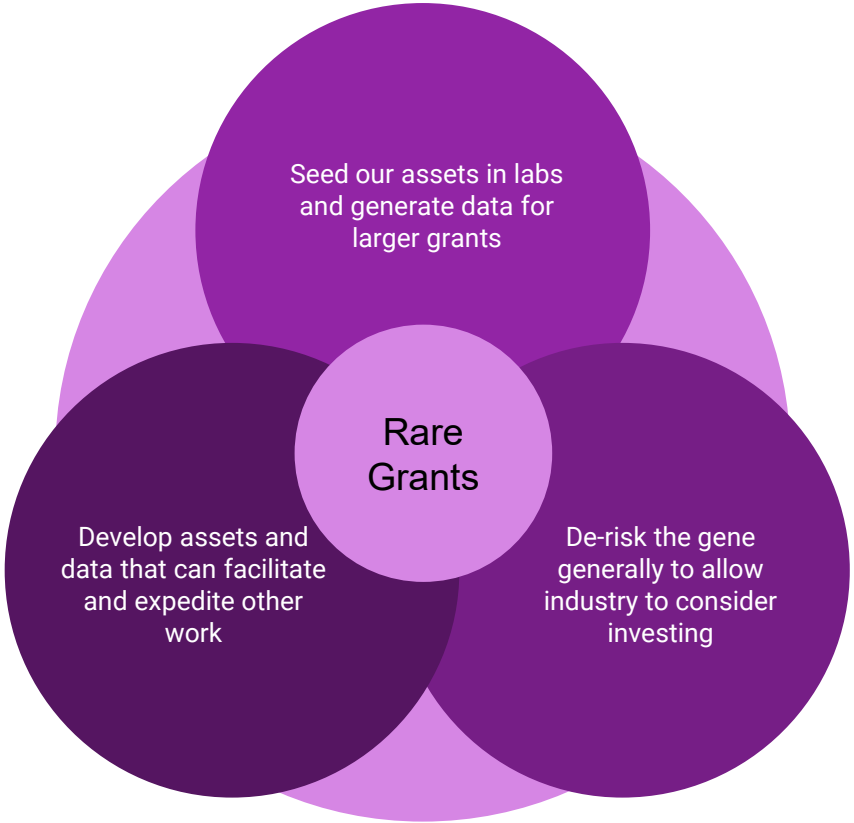
Our capital is rare - Seed, Risk tolerant & Catalytic

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 de-risking 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.



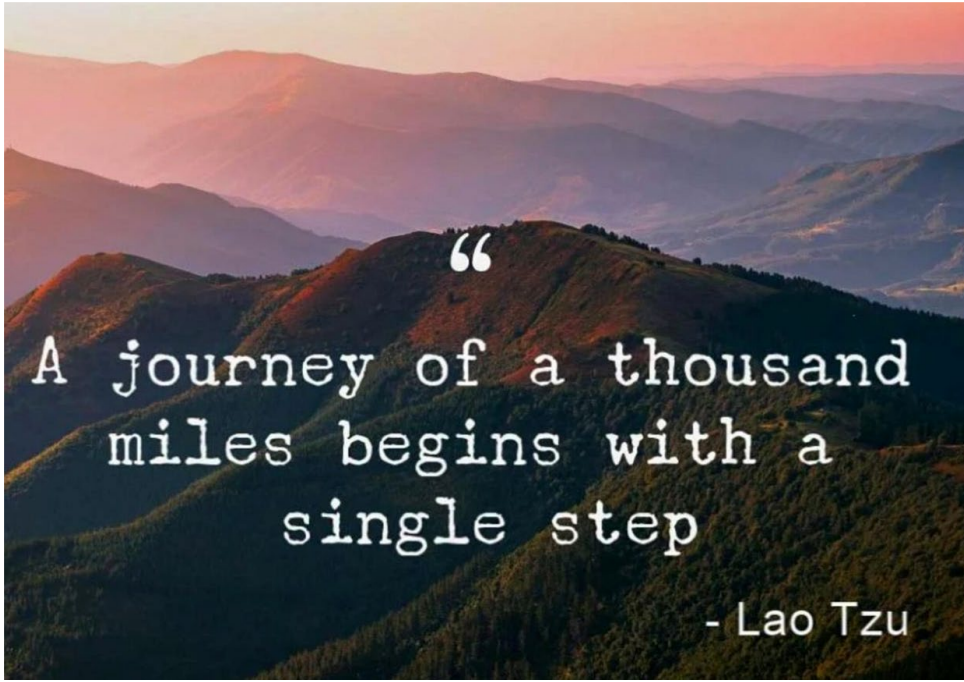
e.g. Your work increases your scores on tables like this.

Disease Name	US / EU Prevalence	Target Gene	Gene Size	Inher.	Onset	Tx Mech.	Nat. Hist.	PC Mod.	Clin. Dev.	Pat. Org.	SZ	ID	MD	Clin. Need	Mkt. Opp.	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	4,032	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
KIF1A 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	<6 m	5	5	4	5	5	5	5	4	5	5	Profound ID, DD with resistant epilepsy & autism
KCNQ2 Encephalopathy	25,467	KCNQ2	2,619	AD	<1 m	4	4	4	4	5	5	5	5	5	5	Severe ID, DD and neonatal onset epilepsy
SLC6A1-Related Disorder	20,603	SLC6A1	1,800	AD	<4 y	5	4	2	4	5	4	4	3	4	5	Moderate ID, epilepsy with regression
CDKL5 Deficiency Disorder	16,868	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	12,366	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	7,776	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

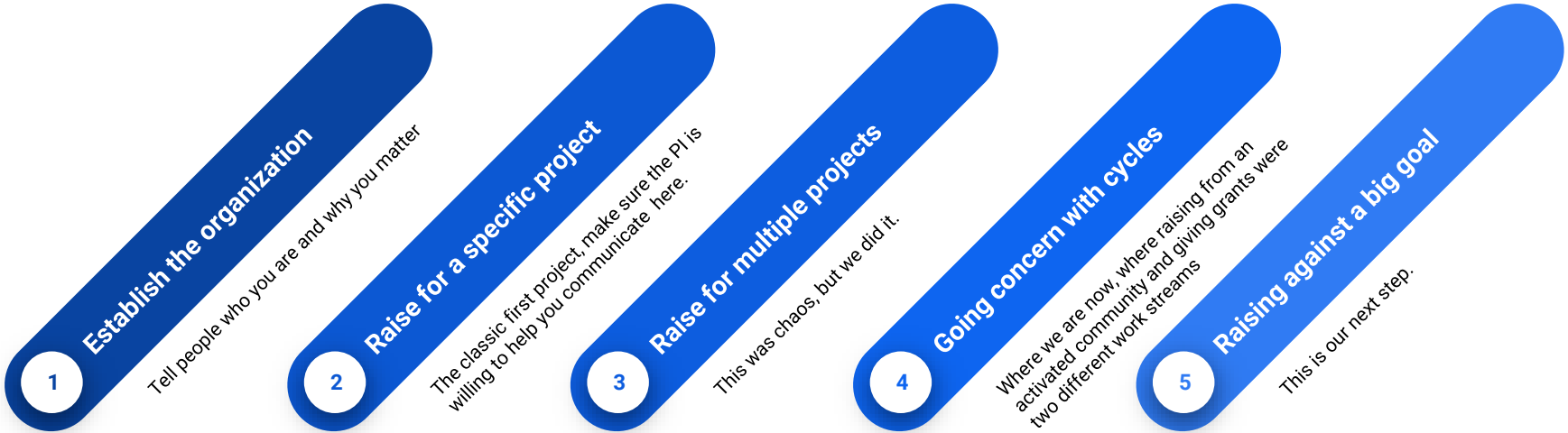
Language & culture matters: “*We can fill an unmet need together*” vs “*I need to raise $100k*”.

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.

CISION PR Newswire — Send a Release

SynGAP Research Fund Announces Grant to Heller Lab of Neuroepigenetics

SynGAP Research Fund — Collaboration. Transparency. Urgency.

NEWS PROVIDED BY
SynGAP Research Fund →
19 Nov, 2020, 09:21 ET

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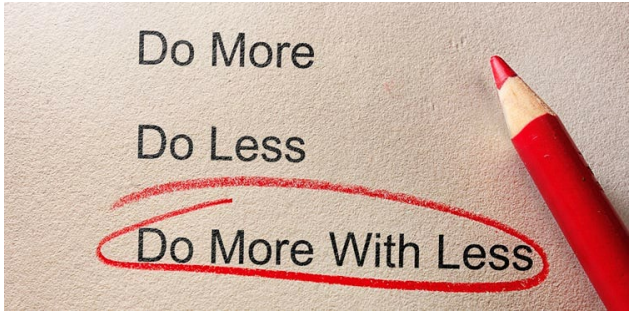
PALO ALTO, Calif., Nov. 19, 2020 /PRNewswire/ -- The SynGAP Research Fund (SRF) announces a new research grant award to the Heller Laboratory of Neuroepigenetics in the Perelman School of Medicine at the University of Pennsylvania. The lab will receive $130,000 over two years towards funding a postdoctoral fellow who will focus on epigenetic regulation of SynGAP1.

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.