

CURE VCP DISEASE



ANNUAL REPORT

2022

LETTER FROM THE CEO

Reflecting on the trajectory of 2022, I am deeply moved by the enduring resilience and tenacity of our community. Together, we have surged forward, making significant strides in our path toward understanding and, eventually, discovering a cure for VCP disease.

A year ago, I shared a moment from our VCP Scientific Conference where a researcher humorously suggested we should focus on what VCP doesn't do, a nod to the profound complexity of this disease. Today, that sentiment drives us not as a reminder of the unknown but as a beacon towards uncharted territories awaiting our discovery.

As both a patient and your fellow advocate, I share the urgency for accelerated research. Our purpose at Cure VCP Disease has been to create an environment where scientists thrive, their work is championed, and they are equipped with the necessary resources. Our continuing efforts to foster collaborative relationships between institutions and industries and to expedite scientific studies have been met with success this past year, and for that, we are grateful.

The embodiment of this collaborative and accelerated approach was our transformative Natural History Study with Nationwide Children's Hospital. In record time, patients were enrolled and active in this study, and within months, we gleaned precious insights into the progression of VCP disease. This endeavor wasn't just a standalone achievement; it inspired further initiatives, leading to our collaboration with Casimir.

Our efforts have not gone unnoticed. The Chan Zuckerberg Initiative's Rare As One Project recognized our success and provided us with a significant grant, a testament to the importance of our work. This generous funding has empowered us to propel our international, patient-led research network further than ever before.

In 2022, we saw the strength and heart of our community reflected in every milestone. Our annual Patient and Care Partner Conference brought us together, united us in our journey, and fueled our collective commitment to eradicating VCP Disease. Our BBQ and Cider Celebration, aside from being a grand reunion, raised significant funds, which will be instrumental in our research and outreach efforts.

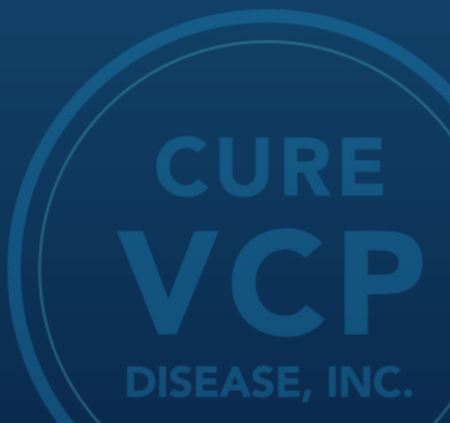
On the horizon, we see promising opportunities for growth, learning, and discoveries that may change the course of VCP Disease. Your unwavering support and shared determination fuel our work, and together, we'll continue to build on the progress of 2022.

Here's to an invigorating and hopeful 2023. Our journey continues, and it is a privilege to traverse this path with each one of you.

With gratitude and hope,



NATHAN PECK
Founder & CEO



CURE
VCP
DISEASE, INC.

OUR MISSION

Unite and fund research while building collaborations and supporting patients within the VCP global community.

OUR VISION

Ensure the availability of treatments and cures while improving the quality of life for patients and families.

BOARD OF DIRECTORS

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Allison Peck - Treasurer
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STAFF

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Leah Miles, Contractor
Director of Partner Development



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Columbus, Ohio

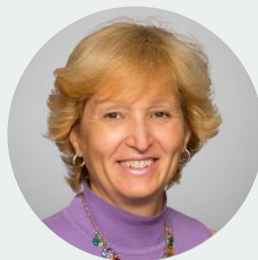
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Daniela Brunner, PhD
Chief Innovation Officer
PsychoGenics

ABOUT US

WHAT IS VCP DISEASE?

VCP disease is a genetic disease that can affect the muscles, bones, nerves, and brain. Individuals with this condition typically develop symptoms in mid-adulthood and may only exhibit one symptom.

This condition is inherited in an autosomal dominant pattern, meaning an affected individual has a 50% chance of passing the VCP mutation to a child. Genetic testing is the best way to diagnose this condition.

Symptoms vary from person to person, even among family members. It is unknown how many people are affected by this condition, but it is extremely rare.

Patients can work with a team of doctors and therapists to develop an individualized, comprehensive care plan to screen and treat the various symptoms that may develop over a person's lifetime. Currently, there is a treatment for Paget's disease of bone, and treatments for other symptoms are also in the pipeline. A care plan for a patient can include physical and speech therapy, prescriptions for mobility aids, monitoring for other diseases, and support for respiratory, pain, and mental health symptoms. These supportive therapies may improve quality of life.

This is no cure yet, but we're working on it!

ABOUT CURE VCP DISEASE, INC.

Cure VCP Disease, Inc. is a patient advocacy organization driving efforts to discover a cure for neuromuscular and neurodegenerative diseases associated with VCP, including IBMPFD, dementia, Parkinson's, CMT, and ALS. We encourage patients and doctors to connect with us. We are committed to collaborating on research and advocacy initiatives and helping patients and their families. Finding a cure is dependent upon the continued generosity of supporters like you. Your support enables us to look beyond our challenges and focus on our fight to find a cure. Thank you for being a part of our journey.



PATIENTS & FAMILIES

We offer connection with other patients and families, research opportunities, and information, such as care guides, support groups, physicians, therapists, and more.



RESEARCH

By making research tools available and fostering collaborations with scientists and researchers worldwide, our Research Network is driving the development of innovative treatments for VCP disease.



CLINICAL CARE

We are a resource to learn more about VCP disease, clinical diagnosis, treatments & therapies, genetic testing, and research.

2022 ACCOMPLISHMENTS

JANUARY

- VCP Standard of Care Published
- 36th & 37th Online Happy Hours

MARCH

- MDA Science & Clinical Conference in Nashville
- 40th & 41st Online Happy Hours

MAY

- Webinar with Dr. Kimonis
- VCP Scientific Focus Group
- 44th Online Happy Hour

JULY

- 2nd VCP Patient & Care Partner Conference
- Hired Leah Miles
- VCP Scientific Focus Group
- 46th Online Happy Hour

SEPTEMBER

- 49th & 50th Online Happy Hours

NOVEMBER

- Completed 1 Year of Casimir Natural History Study
- Created Cure VCP Disease Drug Discovery Scientific Advisory Board
- VCP Scientific Focus Group
- Webinar with Dr. Els Bakker
- 53rd & 54th Online Happy Hours

FEBRUARY

- Testified before Georgia HHS Committee
- Rare Disease Week Held Virtually
- VCP Scientific Focus Group
- 38th & 39th Online Happy Hours

APRIL

- Hired Dr. Armelle Pindon
- Completed 1 Year of Nationwide Natural History Study
- VCP Scientific Focus Group
- 42nd & 43rd Online Happy Hours

JUNE

- Premiered Cure VCP Disease Animated Video
- Chan Zuckerberg Initiative Annual Meeting
- Webinar with Dr. Korb
- 45th Online Happy Hour

AUGUST

- Inaugural Annual Fundraiser in Atlanta
- VCP Scientific Focus Group
- 47th & 48th Online Happy Hours

OCTOBER

- Webinar with Tammy Scott
- VCP Scientific Focus Group
- 51st & 52nd Online Happy Hours

DECEMBER

- Webinar with Kristin Draeger
- VCP Scientific Focus Group
- 55th Online Happy Hour

ACCOMPLISHMENT HIGHLIGHTS

VCP Disease Standard of Care Publication

The “Development of a standard of care for patients with valosin-containing protein associated multisystem proteinopathy” was published in the Orphanet Journal of Rare Diseases on January 29, 2022.

Allison Peck was the second author of the publication and led the effort by coordinating 50 global clinicians to gain consensus on how to handle VCP disease patients.

[Access Publication](#)

Chan Zuckerberg Rare As One Project Grant

Cure VCP Disease received a grant from the Chan Zuckerberg Initiative (CZI) Rare As One Project.

The Rare As One Project is a key initiative by CZI, aimed at uniting patient communities with researchers and clinicians in the shared goal of addressing rare diseases. Leveraging an innovative approach, the project empowers patients and their advocates to play a vital role in advancing scientific breakthroughs.



Armelle Pindon, PhD
Chief Scientific
Officer



Leah Miles
Director of Partner
Development

The CZI grant enabled us to welcome two esteemed colleagues to the Cure VCP Disease team: Armelle Pindon as our Chief Scientific Officer, and Leah Miles as Director of Partner Development. Their combined expertise has proven instrumental in deepening our scientific understanding and expanding our collaborative network.

St. Louis Patient Care Conference - 60+ Attendees

In July 2022, we hosted the Cure VCP Disease Patient and Care Partner Conference at the Westin in St. Louis. Bringing together patients, care partners, family members, the gathering served as a beacon of hope and inspiration for our VCP community, leading VCP researchers in a shared endeavor to illuminate our understanding of VCP Disease.

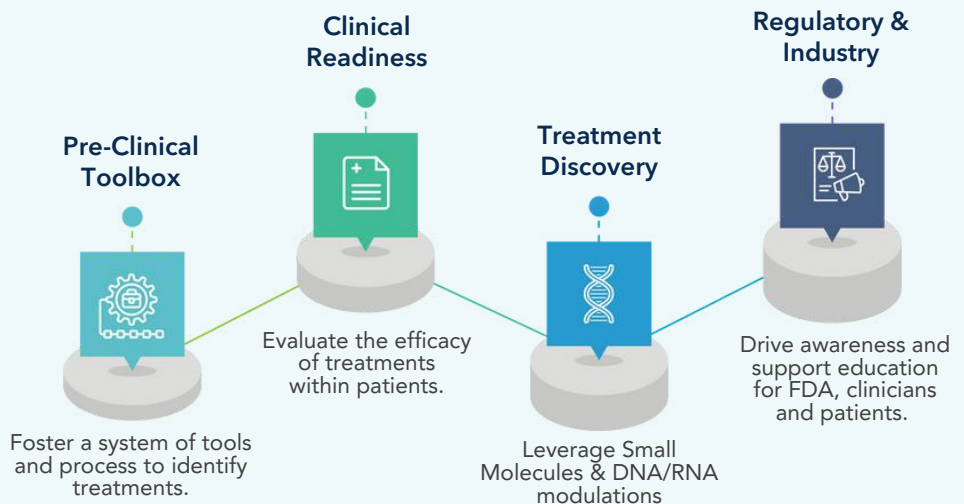


ACCOMPLISHMENT HIGHLIGHTS

Goal: An Ultra Rare Disease Strategy for Treatment Discovery & Clinical Trial Readiness

Our goal is to develop and apply a strategy to understand the gaps, remove the roadblocks, and fulfill the requirements needed for drug discovery and treatment development.

Learn more about our research strategy:
<https://www.curevcp.org/researchagenda>



Completed 1st Year of Natural History Study

Nationwide Children's Hospital enrolled nearly 30 patients with VCP disease in the first year and collected significant data and observations through in-clinic and remote visits.

We look forward to continued participation in year 2 of the Cure VCP Disease study, which will ultimately lead to the largest and most comprehensive functional measures study of VCP disease.

Presentation of Collaboration with Nationwide at European Neuromuscular Conference Workshop (ENMC)

Nathan and Allison Peck traveled to Amsterdam, Netherlands to present on the role of patient advocacy organization-assisted research. Representatives from 10 countries across 5 continents attended the workshop.

[Read Workshop Report](#)



ACCOMPLISHMENT HIGHLIGHTS

Created Drug Discovery Scientific Advisory Board

Complementing our Medical Advisory Board, Cure VCP Disease launched a Drug Discovery Scientific Advisory Board.



Tsui-Fen Chou, PhD
Caltech



Michelle Arkin, PhD
UC San Francisco



Donna Huryn, PhD
University of Pittsburgh

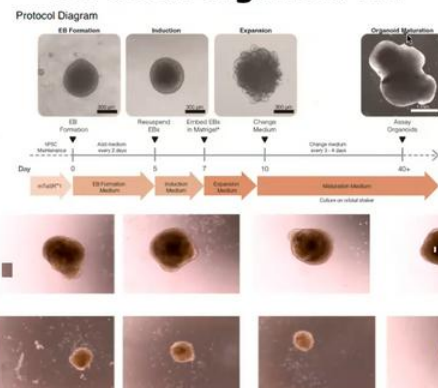


Daniela Brunner, PhD
CIO, PsychoGenics

Hosted 8 VCP Scientific Focus Groups

Bringing together researchers and scientists to discuss VCP, the Scientific Focus Groups drive continued collaboration and insights among researchers.

Making mini brains using stem cell cerebral organoid kit



Premiered a 3-Minute VCP Disease Educational Video

Cure VCP Disease funded a 3-minute animated video to help educate families and researchers about VCP disease. The video has more than 1,000 views!

Watch Video:

<https://go.curevcp.org/video>



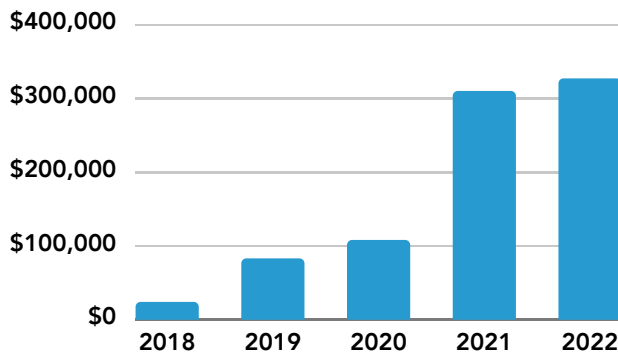
Raised \$65,000 at Inaugural Cure VCP Disease Gala

Through the combined efforts of all attendees and contributors, we were delighted to raise an impressive total of \$65,000. These funds represent more than just a number. They signify the support of our community, the strength of our endeavor, and our unwavering commitment.



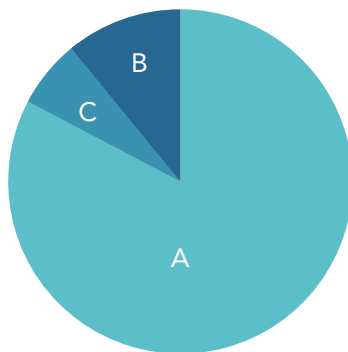
OUR DONORS MAKE AN IMPACT

FUNDS RAISED BY YEAR



2018	\$23,000
2019	\$82,000
2020	\$107,000
2021	\$309,000
2022	\$326,000
Total Funds Raised	\$847,000

EXPENSES



A. Programs	\$215,516
B. Fundraising	\$28,443
C. General & Administrative	\$16,641
Total Expenses	\$260,600

2022 GRANTS RECEIVED

Chan
Zuckerberg
Initiative 

Rare
As
One



\$200,000

Year 2
**Capacity
Building**

THANK YOU FOR YOUR SUPPORT!

CONTACT INFORMATION



229.410.9573



info@curevcp.org



1302 Watson Blvd, #1015, Warner Robins, GA 31093



CURE VCP DISEASE

Cure VCP Disease is a 501(c)(3) non-profit organization.