Our Mission:
Improve the lives of those affected by CHAMP1 disorders through research, effective treatments, public awareness, early detection, family support and patient advocacy.

2021-2024
Goal 1. Raising 100k in 2021 and at a minimum doubling our revenue yearly. 300k by 24

- Starting a peer to peer fundraising Campaign. Utilizing Give butter or similar platform to manage virtual fundraising campaigns, donors and marketing.
- Adhering to consulting for peer to person fundraising direction(give butter or funraise J. Wheeler) Bajro as head of marketing.
- Provide families with basic education and ideas to fundraise
- Participation from vested families to host 1-2 in person fundraising events and put on the calendar.
- Encouraging CHAMP1 family and friends to donating monthly, letter and video announcement to start campaign
- Expanding B2B annual or monthly donors, having detailed proposals to what they are investing into.
- Larger corporations and pharma with proposals and achievements for grants and investments
- Continue to apply for grants through various organizations.

Goal 2. Advancing research from basic to translational

- Having cell lines and mouse models phenotyped
- IPSC characterization( T. Johnson) and differentiation (H. Hakonarson)
- Provide families with education and tools to participate in coriell and CHOP, Fund these 2 projects if needed or fill in the gaps
- 50 lcl lines of diversity, 20, Fibroblasts, 40 IPSC through CHOP
- Keep working with uab, 123 Genetix and potential organizations to utilize AI for druggable targets and simulated probabilities.

Goal 3. Develop partnerships with biotech

- Have basic science completed )characterization, body chemistry.
- Tools freely available
- Registry detailed with contactable and ready community, Gather questions and registry information from experts.
- Researchers open and ready to collaborate
- Need a clinician who can serve as a center of excellence or expert in CHAMP1 (Wendy?)
- Continue to prospect new Dr’s for new technology and innovation, reach out to experts with similar molecular biology
- Put out RFA’s to attract new researchers
- Have sit down with FDA, engage about patient advocates desires and meaningful change

Advancing Research
Awaiting results RNA sequencing, proteomics, computer screening, mouse models
- Abundance or reaction to uptick in protein related to cancer? Need a mouse model with a turn on and off gene to identify if CHAMP1 has recovery potential? As families with terminal loss have high functioning children with absence of gene, is mutation creating harmful protein?
- Working with existing advisory boards and CHAMP1 researchers to support them, identify best next steps, future projects and advice on what avenue to pursue next.
- Continue reading, identifying researchers in related fields or sciences that may have interest in characteristics or scientific processes similar to CHAMP1.
- Build registry as a means to attract young post docs on taking on new projects.
- Advertise biobank samples, fibroblasts, IPSC, blood samples
- Work with nutritional companies, Doctors willing to investigate body chemistry, do testing ect.

Possible Treatment Options
- Find small molecule to uptick regulation of functional allele.
- Gene therapy
- Gene replacement
- Exon skipping (Read through compounds)

Public Awareness
- Delegate social media. Building following through family and friends involvement. Regularly, at least weekly, sharing information and educating.
- Creating quantities of education materials, awareness cards, bracelets, pamphlets, flyers.
- Commit to hosting a table at least 1 conference a year.
- Commit to 2 larger awareness related events locally.
- Encourage, support, and provide the CHAMP1 community with the tools and resources to represent their local community.
- Continue to contact local media, attempt to make shorter videos or attempt to go viral and attract larger audiences.
- Find 3 celebrities to make short pitches for CHAMP1.
- Brand CHAMP1 by perfecting logo, #CHAMP1ON.

**Early Detection**
- Create materials and agenda to attend conferences to give out education materials
- Utilize contact list to reach out to diagnostic companies to add CHAMP1 to the panel.
- Partner with a diagnostic company to provide whole exome sequencing (WES) if not available through insurance.
- Create flyers with CHAMP1 symptoms and ask diagnostic centers to post and evaluate potential candidates.
- Pay for databases to spread the word to clinicians/diagnostic and clinical geneticists.
- Continuing advancing awareness missions with the medical community at every opportunity.

**Family support and advocacy**
- Educate the community through answering questions and providing important information in one centralized location (i.e. website).
- Create relationships with CHAMP1 members and families, identify needs and receive feedback.
- Equip families with resources and materials needed to advocate for themselves.
- Capitalize on family meetings by recording information, speakers, and educational materials. Post them to a website and Facebook group.
- Ask invested doctors to record relevant information for reference of existing and new families.
- Be the primary point of contact for families.

**CHAMP1 Strategic Roadmap**

**Immediate Goals**

Family meeting June 28-30th (see google doc)

**Short Term Goals (accomplished by year end 2019)**

**May 2019: Bylaws**

Establish Foundation Board, Officers, and Committee Chairs:

(See Bylaws Document)

President: Katis D'Angelo
Vice President: Jeffrey D'Angelo
Secretary:
Treasurer:
Each family unit serves as a board member. 1 vote per family.
Committee Chairs: Financial Committee, Social Media, International
Head Committee Chair: Vice Chair: Members of the Committee:

June 2019: Family Meeting

August 2019: KO, Founders, and LJ/JJ mice created and phenotyped (JAX/UC DAVIS/ROD in process of creating mice.

We need mice because this is a tool for researchers, it can create interests and be a crucial part of drug development.

December 2019: Natural History Study: At least 20 families registered (w/ SimonsVIP)
Currently 8 registered: We need clinical data to eventually start trials.
We need to have at least 20 families registered in the natural history study because a natural history study will create a database of CHAMP1 information which allows researchers to gain an overall understanding of a clinical genotype/phenotype relationship.
- Protocol in place for new families, implemented through our community support chair.
- Education and emphasis to existing families of the importance and benefits to having a completed registry.
- Utilize Experts in the field to assist with encouraging the community to participate.

December 2019: Biobank: At least 10 families blood and skin biopsies biobanked (w/ Coriell)
Currently 2 families are biobanked.
We need a biobank because it will allow researchers to obtain cell lines e.g. Fibroblasts, IPSC, Lymphoblastoid which will provide researchers tools to meet CHAMP1 research objectives. Strategy is the same as Natural history study.
- Fibroblasts for drug re-purposing (small molecule)
- Phenotype IPSC
- “Mini Brains”

December 2019: Fundraising to have budget of $100,000 by year end
Currently: $20,000
Establish yearly operating budget
We need to raise $100,000 by year end because it will provide an operating budget which will allow for allocation of funding toward future research opportunities, awareness, and patient support and advocacy. (See Budget)
- Establish and grow our fundraising chair committee.
- Create a 2020 Fundraising calendar of events.
- Commit to learning and applying to new grants at least 1 a year.

Year 1-3: Increase overall budget income 75% of previous years total.
- calendar
- commitment from families
- larger marketing efforts
- Tap into grants

1-Year 3: Establish a team/group of scientists committed to working together, sharing data and collaborating preliminary data including but not limited to: RNA sequencing (Lessel and Hakonarson), phenotyping of mouse models (Rodney), phenotyping of cell lines (McConell? Bodammer?), proteomics (Lessel), and body chemistry (Jill Weimer)

After baseline data is produced, work with scientific advisors to select most effective scientific approach to develop a pathway to treatment

Year 5: Have centers of excellence established in countries with the highest population of CHAMP1.
- Established standard of care for CHAMP1(precision medicine)
- participating doctors or institutions
- ongoing coordinated clinica/ natural history study