

## External Evaluations – 2020

### Chan Zuckerberg Initiative (CZI) Award

PSC Partners is honored to have been one of the thirty rare disease organizations to be selected from 287 applications from 43 states. We were selected based upon our patient led approach. Here is a link to the article about the program and awards: <https://chanzuckerberg.com/newsroom/chan-zuckerberg-initiative-awards-13-5-million-to-drive-progress-against-rare-diseases/> Here is a link to the video and the list of recipients: <https://chanzuckerberg.com/science/programs-resources/rareaseone/>

### Great Nonprofits

We were designated as a Top-Rated 2019 Great Nonprofit.

Here is the link to the reviews: <https://greatnonprofits.org/org/psc-partners-seeking-a-cure>

### Patient-Focused Drug Development (PFDD) Forum – 10/23/2020

PFDD Abstract: The U.S. Food and Drug Administration (FDA) has multiple mechanisms for its regulators and staff to interact with patients – but none quite like its novel Patient-Focused Drug Development (PFDD) initiative. FDA established the PFDD initiative to more systematically obtain the patient perspective on specific diseases and their currently available treatments. Since the initiative’s inception in 2012, FDA has held 24 PFDD meetings, covering a range of disease areas and hearing directly from thousands of patients and caregivers. FDA’s PFDD meetings have also provided key stakeholders, including patient advocates, researchers, drug developers, healthcare providers, and other government officials, an opportunity to hear the patient’s voice. The lessons learned include but are not limited to specific experiences that matter most to patients, patient perspectives on meaningful treatment benefits and how patients want to be engaged in the drug development process. FDA recognizes that FDA-led PFDD meetings alone cannot address the gaps in information on the patient perspective. Further enhancing the incorporation of the patient’s voice in drug development and evaluation continues to be a priority for FDA.

PSC Partners Seeking a Cure presented to the FDA and the PSC community on 10/23/2020. Here is some feedback from the meeting.

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This was a great workshop. I had to stop listening at times because I was crying so hard and had a bad headache. This is a terrible disease, I hate it.

I thought your closing comments were great! You are amazing.

FDA Attendee

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Wow. I just have to send out a HUGE thank you to those who were a part of organizing and pulling together the PFDD Forum. I am so grateful for ALL of the people who took part in the MANY hours collecting data, creating educational slides, finding knowledgeable speakers, monitoring the forum, and much more. What an incredibly professional, moving and informative meeting- I am at a loss of words to tell you how emotional it was to listen to everyone’s stories and I truly feel that the PSC voices were heard loud and clear today!! Thank you Leah, Jen, Fred and Willie (and to all the participants) for sharing your touching, painful and passionate stories in order to educate those listening about the symptoms,

fears and hopes of our PSC family. What a mighty force this group is when we work together- I feel honored to know you all!

There will be a treatment/cure with this team at the helm- I can feel it!

PSC Partners Board Member/PSC parent

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The session today was poignant and inspiring and wanted to congratulate you on putting together such an impactful meeting despite all the logistical challenges of COVID-19. Thank you for all the work you are doing to push the field and bring the patient experience to the forefront of research – this is so important. I know there were more than a few of our colleagues on for at least some period today but we plan to share this more broadly with the company once a recording is available.

VP of a pharmaceutical company

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I attended the all day forum and give all involved an A+++.

The sessions were well run. Very moving individual testimonies followed by panel discussion and call in's pertinent to the topic.

The moderators kept it all on point and on time.

Congratulations.

PSC Caregiver

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I was able to listen to some of the PFDD Conference. It was very moving and wonderfully done. The organization, focus, flow and content was excellent and certainly motivating for my group to hear the stories of so many people in the PSC community.

Congratulations and so happy to be working with such a great group.

PSC Doctor

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Yesterday's forum was outstanding.

It was crystal clear that the hosts, presenters, speakers and participants gave everything they had. I'm so thankful to those who went through the very difficult process of sharing their personal stories - the impact of those stories was profound. I sat at my computer for the entire six hours, glued to my chair. It was devastating to see the extraordinary suffering but also very proud of this community and it's selfless commitment for a future treatment and cure. To see the decline in health of any member of our community is overwhelming to me and surely had an equal impact to those present yesterday.

I can't imagine how much work went into the day and it showed. It was a master class. Thank you to your team and everyone that had anything to do with the day.

I'm sure you're being bombarded with thank you's and praise and you deserve each and every one of them. It was as if you poured everything you have ever learned in your lifetime into this moment.

The mission of the forum was clearly achieved (if our need wasn't clearly identified yesterday, that individual must not have a heart) and as you said, this community is ready to help in every way we can.

Thanks again for all you do - we are grateful beyond belief.

PSC Patient

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