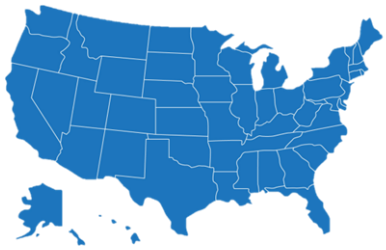
PATIENT VIEW
UNDERSTANDING PATIENTS

BPC/1 APPENDIX: SUPPLEMENT TO THE *BEING PATIENT CENTRIC* TOOLKIT

HOW PATIENTS CAN BE MORE CLOSELY INVOLVED IN THE RESEARCH AND DEVELOPMENT OF DRUGS: US EDITION

THE VIEWS OF 104 US PATIENT GROUPS
APPENDICES, FEEDBACK AND COMMENTS

PUBLISHED NOVEMBER 2018



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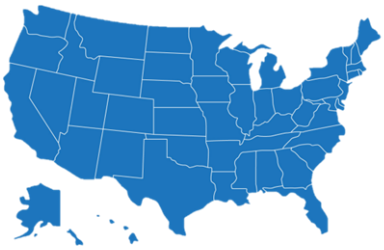
FEEDBACK FROM US PATIENT GROUPS:

* All comments have been intentionally anonymised



FEEDBACK FROM US PATIENT GROUPS:

- STATE OF PATIENT ENGAGEMENT IN R&D
- CHALLENGES TO PATIENT ENGAGEMENT IN R&D
- HANDS-ON EXPERIENCES OF FDA's PFDD; HOPES AND CONCERNS
- RECOMMENDATIONS TO IMPROVE PATIENT ENGAGEMENT IN R&D

STATE OF PATIENT ENGAGEMENT IN R&D

Participating in research and development

- "While we are not the lead in advancing research, we are a participatory lead in research projects related to genetic mutations associated with ours." [Rare-disease patient group]
- "We also have a research registry through PCORnet (the National Patient-Centered Clinical Research Network), and partner with the University of South Florida to facilitate research." [Cancer patient group]
- "Our research has been focused on incorporating patient values into the treatment decision process from the beginning." [Cancer patient group]
- "We have also participated in FDA meetings, providing an unbiased patient perspective on end points and outcomes." [Cancer patient group]
- "We helped develop endpoints for two clinical trials." [Neurological-conditions patient group]
- "We have worked to improve [how] the patient perspective is heard in clinical-trial recruitment settings." [Gastrointestinal-conditions patient group]
- "We also support a patient/family registry, that has the lay person enter their information, with the option of an online informed consent, to learn about possible research studies/trials that could be of interest. But we have a long way to go in making that an efficient process." [Neurological-conditions patient group]
- "In past years, we have directly participated at the FDA by testifying, and now writing letters. Listening to other patient advocates." [Cancer patient group]
- "Our organization focuses almost exclusively on public policy—Congress, the NIH/NCI and the FDA." [Cancer patient group]

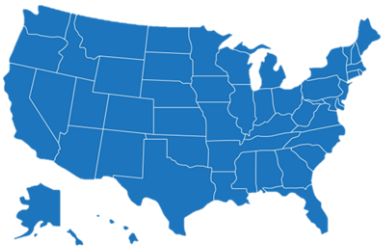
Funding research

- "We fund researchers working at other non-profit institutions. They do not work for our organization, but do report to us on their work, as part of the grant award process." [Rare-disease patient group]
- "We raise funds, and issue grants to researchers. We also coordinate conferences attended by patients and researchers, and foster dialog." [Cancer patient group]
- "We are working with a medical researcher, taking an exciting pain-and-inflammation -reducing medication to clinical trials in 2019." [Neurological-conditions patient group]
- "We run a number of grant programs that cover basic science, clinical research, biomarkers and clinical trials." [Neurological-conditions patient group]
- "We have only worked with one CRO [Contract Research Organisation] at present. It was/is a positive experience. We also have an established dialogue with clinical research networks, biotechs, big pharma, the FDA, the NIH, and many of our non-profit peers, in how we can make drug development more patient friendly, as well as more grounded in the patient's needs for maintaining independence for as long as possible, and acceptable daily quality of life." [Neurological patient group]
- "Undertaking medical research. We serve as PI [Principal Investigator] on several multi -center studies, which are run through those centers and their IRBs [Institutional Review Boards]." [Respiratory-conditions patient group]

THE NATURE OF THE RELATIONSHIPS BETWEEN PATIENT GROUPS AND PHARMA ON R&D IN GENERAL; AND IN RELATION TO PFDD

Specific comments

PATIENT-GROUP COMMENTS:	LEVEL OF IMPORTANCE
Teach the C suite [top senior executives] and counsel to trust patients' involvement.	2
Do something about patient access to their drugs. A broken system. 'Free' or 'reduced-cost' drugs only keep the prices up, and access ultimately down.	3
Stop funding doctors on trials that waste time, cost, and government funds. We have seen too many repetitive studies. Studies must focus on elders with Parkinson's.	3
Engage patient groups on the public-policy development process at the FDA and the Hill.	3
Actively promote and support disease-awareness and prevention efforts.	3
Money, and putting patients on their consulting and advisory board with a substantial fee, so that patients can afford to give them the information and access to the cancer patients they need for clinical trials.	3
Look at the long-term effects in current cancer treatments for children, and adolescents and young adults (AYAs), and come up with better and safer treatments.	3
Allow men into breast-cancer trials, or open a trial with them alone.	3
Actually learn the diagnostic criteria for our condition, and train screeners (who are usually not specialists in our condition) to properly screen us. Too many of us are improperly disqualified, because our health condition is not understood by screeners.	3
If there is a way to involve patient groups in the process, inform these people and groups as to how they can help.	3



DR. SCOTT GOTTLIEB, THE FDA COMMISSIONER, HAS EMPHASISED THE NEED TO INCLUDE MORE 'REAL-WORLD' EVIDENCE [RWE], AND REDUCE THE EMPHASIS ON CLINICAL-TRIAL EVIDENCE, IN THE REGULATORY APPROVAL OF DRUGS.

IN 2018, THE FEDERAL GOVERNMENT DEDICATED $100M TO DEVELOP A SYSTEM CAPABLE OF COLLECTING SUCH 'REAL-WORLD' DATA.

US PATIENT GROUPS WERE ASKED IN THE PATIENTVIEW AUGUST-OCTOBER 2018 PFDD SURVEY TO GIVE THEIR OPINION AS TO WHETHER THEY BELIEVE REAL-WORLD EVIDENCE WILL BE BETTER THAN CLINICAL-TRIAL EVIDENCE IN MEASURING THE EFFECTIVENESS OF PHARMACEUTICAL PRODUCTS

CLINICAL TRIALS V. REAL-WORLD DATA

NO, REAL-WORLD EVIDENCE IS LESS EFFECTIVE [4%]

PATIENT-GROUP COMMENTS

- "Double-blind clinical trials are the only means to truly determine cause and effect. [Cancer patient group]
- "No effective controls in 'real-world data'. They are not objective." [Neurological patient group]

DO NOT KNOW [5%]

PATIENT-GROUP COMMENTS

- "Depends on how it is designed, and how much weight/resources it takes away from clinical trials. In neuro-endocrine tumor (NET) cancer, Europe could give us lots of real-world data on drugs in use there, but which were much delayed here by necessary clinical-trial requirements. I can see a place for 'real world' in that case. But, it should not take the place of science/trials altogether. Safety should not be compromised. Case-by-case basis?" [Cancer patient group]
- "I need to learn more about this. In theory, it makes sense, but I have not heard all the sides on this issue, as yet. Hope to hear more." [Cancer patient group]
- "It's not an either/or situation. Both clinical-trial and real-world evidence need to be used for evaluation, as they often offer complementary views. One example would be that registries maintain contact with the patients on a trial, even after it has finished. So, for diseases with longer survival times, that have drugs with higher efficacy rates, raw data can be observed on a different time horizon (not bound by the time strictures of the trial)—often giving a more-accurate picture of data like overall survival, and progression-free survival." [Cancer patient group]

PATIENT VIEW

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APPENDIX:
SUPPLEMENT 1 TO THE *BEING PATIENT CENTRIC* TOOLKIT

TO BE READ ALONGSIDE THE MAIN REPORT