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

THE VIEWS OF 104 US PATIENT GROUPS

The first of two supplements focusing on patient engagement in R&D. Both supplements are intended to be used in conjunction with PatientView's evidence-based Being Patient-Centric toolkit (published November 2017)

PATIENT VIEW
UNDERSTANDING PATIENTS

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EXECUTIVE SUMMARY

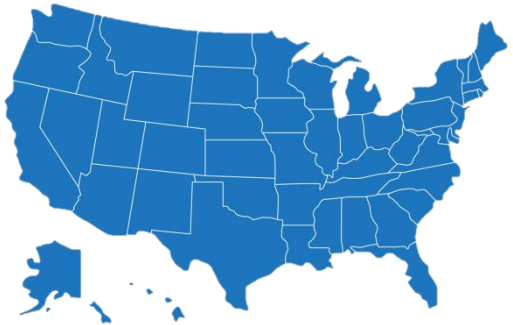
The patient movement worldwide has grown exponentially in reach and influence over the past decades. The movement was kick started in the US back in 1987 when AIDS activists took to the streets in Washington DC, raising the issue of patient rights' to front-page news for the first time. Since those days, patient-group activism and advocacy has mainly been the preserve of patient groups in Europe, under the encouragement of the European Commission.

However, in 2017-2018, US patient groups once again began to take a leading role in shaping their healthcare system. Outside forces have been responsible for the renewed activism—chiefly, the Food and Drug Administration (FDA). The regulator is actively promoting programmes which put patients at the heart of healthcare. One initiative is particularly ground-breaking—Patient Focused Drug Development (PFDD). The PFDD initiative invites and encourages patients and patient groups to participate in the process of drug research and development (R&D) from the onset.

The PFDD initiative could bring significant consequences, not only for the US patient movement, but for those patient groups outside the US which are also taking an interest in what is happening in the country.

About this survey

Because of the importance of the FDA's PFDD initiative, and the heightened activity of US patient groups in drug R&D, PatientView decided to conduct a survey of patient groups in the US on the subject. The survey ran August-October 2018, and this report contains the survey's results.



EXECUTIVE SUMMARY

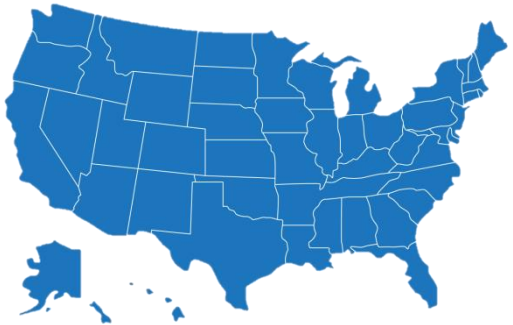
The study's 104 respondent US patient groups were asked about 4 important topics: (1) How patient groups define patient-centric R&D; (2) Their views on the FDA's PFDD; (3) Their views on pharma's performance at being patient centric in R&D; and (4) Their views on the role of 'real-world' evidence versus clinical trials. The report and Appendix analyse the opinions of all of the respondent US patient groups, and, more specifically, the responses from US patient groups specialising in cancer, rare diseases, and neurological conditions.

From the feedback received, the vast majority of the US patient groups responding to the August-October 2018 US PFDD survey consider that activities undertaken during R&D should always incorporate the patient perspective, and 56% of the respondent US patient groups think that the main outcome of engagement should be R&D investment which truly addresses patients' needs.

The majority of US patient groups believe that the best definitions for effective patient engagement in R&D are:

- R&D investment which demonstrates that it truly addresses patients' needs.
- Examining how the output of R&D affects the COMPLETE patient life experience.
- Patient-friendly protocols for clinical trials, and post-trial follow up.
- All parties involved in the clinical-trial process consider the patient perspective.
- Patients are made aware of, and are able to participate in, clinical trials of relevance to them.

However, most US patient groups also believe that much of their understanding of what makes effective patient engagement in R&D is NOT yet happening in the US. Moreover, Only a minority of the respondent US patient groups believe that other healthcare stakeholders are willing to engage with patient groups on R&D activities.



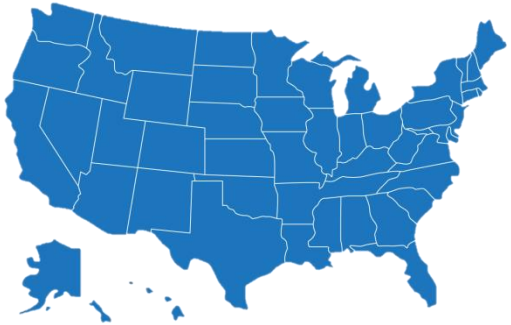
EXECUTIVE SUMMARY

Not surprisingly, then, 98% of the respondent US patient groups declare some sort of interest in the FDA's PFDD initiative (only 2% are not interested). Around one third of the US patient groups have already been involved with the FDA's PFDD initiative in some way. Almost 40% are interested, but not sure how to engage.

HOW SHOULD PHARMA REACT?

Over half of the respondent US patient groups state that they work in general with pharma to improve patient engagement in R&D. 28% of the US patient groups state that they have worked with pharma on the FDA's PFDD initiative. But, clearly, from the many comments received from these organisations, pharma could significantly improve these types of relationships.

The 104 US patient groups were asked to list three optimum ways in which pharma could be more effective at engaging patients and patient groups in R&D. The hundreds of comments were analysed and weighted on the frequency of mentions, to produce a 'wish list' (seen on slide 29). Top of the list, though, is the wish to see patient engagement early and throughout the drug R&D process.



RESULTS – THE DATA

RESULTS FROM THE US –THE DATA

PROFILING:

- Levels of US patient-group engagement in R&D.
- Stakeholder willingness to support US patient-group engagement in R&D.

DEFINITIONS AND VIEWPOINTS:

- How US patient groups define patient-centric R&D.
- US patient groups' views on whether patient-group engagement in R&D is actually happening.

ON THE FDA'S PFDD:

- Familiarity/engagement with PFDD among US patient groups.
- The impact that the FDA's PFDD initiative is having on …
  - ➢ improving patient centricity in R&D;

  … and on the willingness of other stakeholders (including pharma) to …
  - ➢ engage with US patient groups in R&D.
- Main concerns about PFDD.

ON PHARMA:

- The nature of the relationships on R&D between US patient groups and pharma.
- Pharma's main challenges in involving patients/patient groups in R&D.
- The three most-important actions that pharma can take to help PFDD work.

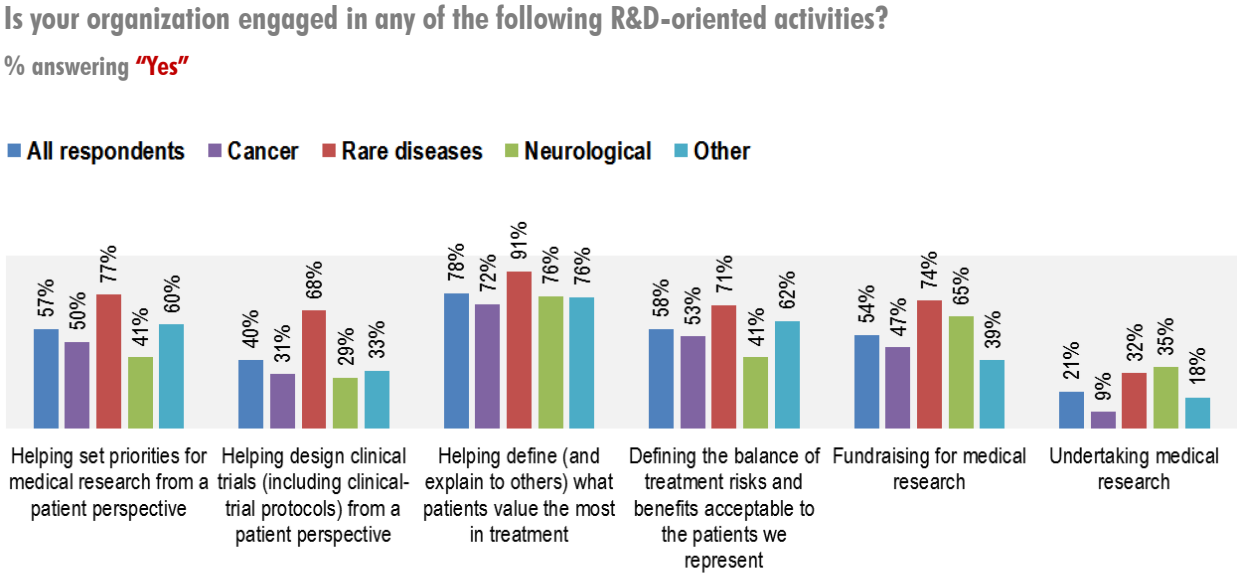
ON CLINICAL TRIALS v. REAL-WORLD DATA:

- Preferred options.

PROFILING: US PATIENT-GROUP ACTIVITIES IN R&D

Levels of US patient-group engagement in R&D

- The study's respondent US patient groups were self selecting. The majority are involved, one way or another, with R&D-oriented activities.
- As many as 78% of the respondent US patient groups believe that they have helped define what patients value most in treatment (figures for cancer, rare disease, and neurological patient groups were 72%, 91%, and 76%, respectively).
- Of all the types of respondent US patient groups, those specialising in rare diseases are the most active and involved in R&D—and in a wide range of ways.



A FEW COMMENTS FROM PATIENT GROUPS:

- THOSE EXPERIENCED IN PFDD
- HOPES
- FEARS

A FEW COMMENTS FROM US PATIENT GROUPS INVOLVED IN THE PFDD PROCESS

"We have had an internally-led PFDD meeting. We have convened a patient-advisory panel within our research consortium to align with PFDD goals. Several published papers have referenced PFDD reports, and we are currently engaging in patient surveys to determine patient preferences for treatment outcomes.

We have presented on the PFDD initiative at conferences, and are currently involved in the planning stages of an externally-led PFDD meeting."

— NATIONAL US NEUROLOGICAL-CONDITIONS PATIENT GROUP

"Our recent experience with hosting a PFDD impressed us on the sensitivity by the FDA to patient issues."

— NATIONAL US CANCER PATIENT GROUP

THE HOPES …

"It gives us a forum to have a dialogue across stakeholders—and, most particularly, brings the patient voice to a room where even the FDA needs to learn more about our rare disease."

— NATIONAL US NEUROLOGICAL-CONDITIONS PATIENT GROUP

"Very interested in working with the PFDD initiative in the near future. We would love to know more about it."

— INTERNATIONAL US NEUROLOGICAL-CONDITIONS PATIENT GROUP

A FEW COMMENTS FROM US PATIENT GROUPS POSITIVE ABOUT THE FDA'S PFDD PROCESS

THE CONCERNS …

"This seems to be a complicated process, and is quite daunting to organizations such as ours."

— NATIONAL US NEUROLOGICAL-CONDITIONS PATIENT GROUP

"It remains to be seen how the FDA will get all its review divisions to buy in to the PFDD process of incorporating the patient voice."

— NATIONAL US RESPIRATORY-CONDITIONS PATIENT GROUP

A FEW COMMENTS FROM US PATIENT GROUPS POSITIVE ABOUT THE FDA'S PFDD PROCESS

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FOR FURTHER INFORMATION, PLEASE CONTACT REPORT@PATIENT-VIEW.COM

Being Patient Centric (BPC) is a 2017 toolkit developed by PatientView with the support of thousands of patient groups, and intended to help pharmaceutical companies become more patient centric.

BPC supplements expand on the core themes developed in the initial 2017 toolkit. Supplements 1 and 2 (November 2018) focus on patient centricity in R&D.

An evidence-based approach lies at the heart of all of PatientView's BPC publications.