PATIENT ENGAGEMENT IN R&D: STILL A CHALLENGE?

BEING PATIENT-CENTRIC TOOLKIT
• THE OPINIONS OF PATIENT GROUPS WORLDWIDE •
DECEMBER 2018

PATIENT VIEW
UNDERSTANDING PATIENTS

FOREWORD

The pharmaceutical industry has, until quite recently, considered its primary customer to be healthcare professionals; those teams and individuals trained and qualified to make decisions on behalf of patients. However, this generally patrician approach to patient care has, in the past few years, been eroded by consumer expectation and knowledge sharing, leading to more informed, confident and empowered patients. There are also wider shifts across the entire system, with clinicians becoming more patient-centric, regulations opening up more patient involvement, payers and insurers being more focused on patient involvement and choice. As a result, a new model is emerging that places patients and carers at the heart of the pharmaceutical business. In essence, those in need have finally become the primary customer, defining their own needs and expectations and helping to set a strategy for pharma investment and activity that goes beyond assumptions made by industry and healthcare professionals on their behalf. Such a shift is a gradual evolution, but still represents a significant challenge for pharma, as it seeks to accommodate regulatory demands, shareholder interests and broader societal challenges—such as a healthcare system's ability to pay for new technology.

In November 2017, PatientView collated insights from over 2,000 patient groups to produce a toolkit for pharma – a 360-degree approach that ensured that any patient-centred strategy was considered from all sides, not just from one specific therapy or functional perspective. As a result, *Being Patient-Centric* was launched and it identified 9 key attributes of patient need. Of these, *patient engagement in R&D*, was consistently rated low by patient groups (in terms of patient satisfaction with activities of pharma).

This finding has prompted further surveys, analysis and research in 2018 to inform two in-depth supplements to *Being Patient-Centric* on the topic of patient-centric drug R&D, undertaken from both a US and global viewpoint. The separate US survey was considered necessary due to legislation passed in 2016 that led the Food and Drug Administration to systematically incorporate into the regulatory framework patient perspectives via the *Patient-Focused Drug Development* (PFDD) programme. The programme is unique because PFDD encourages patient engagement in an enforced, top-down manner. The US report was published separately in November 2018.

The global report, presented here, reflects, in general, the voluntary approaches in patient-centric drug R&D ongoing in the rest of the world.

From the evidence collected for this supplement, it is clear that significant challenges are being faced both by pharma - in adapting to the incorporation of a new stakeholder's views, and in patients and patient groups themselves who do not have as much time, resources and skills to effectively engage with pharma's complex global infrastructure. This supplement looks at those challenges across the life cycle of a product from beginning to end – not only from an evidential perspective, but also through the words of patients themselves, with a comprehensive appendix of direct quotes.

In reviewing this supplement, pharma might reflect that patient engagement has not been at the forefront of R&D activity, and most expertise sits in public affairs, communications or marketing where allowed. As such, focus needs to be placed not only on 'what needs to be done' but also 'by whom', and 'do they have the skills, support and structure to be successful?'

The benefits of effective patient engagement in R&D are discussed in the introduction, and it is hoped that this supplement can help pharma realise these more quickly. Faster approvals, more useful endpoints and better outcomes, as defined by patients themselves, are common goals that can only be of benefit to everyone.

We would like to thank the thousands of patient groups that have made this report possible.

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INTRODUCTION

Patient-centricity is an articulated goal for almost all pharma companies, nowadays. A key component of any pharma company's patient-centric strategy will be (or should be) the effective engagement of patients (and patient groups) in research and development—*as stipulated by patients themselves*[1]. Since 2016, activities in this area have stepped up across the healthcare landscape, with pharma companies, new multi-stakeholder consortia, and even regulators, moving from talk to action. Yet, despite the excited interest, numerous imponderables continue to hinder progress. Industry executives are largely won over to the value of the idea, but are confused about what it involves—and, therefore, how to bring it about. In particular, they are unsure of the benefits of investing in the necessary patient engagement for patient-led R&D.

This latest December 2018 supplement to PatientView's 2017 ***Being Patient-Centric*** toolkit seeks to provide more detailed evidence by reviewing the literature, talking to company executives, and analysing the feedback from patient groups responding to PatientView's 2017 'Corporate Reputation of Pharma' survey (in which patient groups were asked, for the first time, about their experiences with pharma R&D). In addition, during August to October 2018, PatientView conducted a survey of over 100 US patient groups to obtain their views on the Food and Drug Administration's (FDA's) Patient-Focused Drug Development (PFDD) initiative, and on the equivalent activities of research-based pharma. The results of the 2018 US PFDD survey are available as an additional stand-alone ***Being Patient-Centric*** supplement.

A patient group responding to the 2018 US PFDD survey sums up the general view: "*Patients should be involved early, and often, in the entire drug-development process—starting with basic research and continuing through to post-market evaluation.*"

"*The patient-organization submission should also become a firmly-embedded component of the clinical-trial process*", echoes the Thyroid Federation International of Canada, responding to the 2017 'Corporate Reputation of Pharma' survey.

A national pain group from the UK agrees, adding that companies must avoid "*waiting for the drug to come out on the back of a load of assumptions.*"

Some definitions

The process of drug research and development is complex, and its many components can be understood in varying ways, according to different stakeholders. However, from a patient-centric perspective (that is, the patient viewpoint on the subject) drug research and development should be defined as follows:

Research/discovery involves all activities before clinical trials: deciding key unmet needs, outcomes to be investigated, and where patients/patient groups can help identify endpoints. Patient and patient-group contributors can also support ways to improve the patient experience before clinical trials begin.

Development includes all activities once clinical trials begin: starting from the clinical trials themselves, throughout the drug's lifetime, to the point at which the drug/product is mature, and when it leaves the market. Patients and patient groups can help in numerous ways, by assisting with patient recruitment, guiding pricing, providing input on the topic of market access, helping improve patient compliance, and understanding patient experiences after the time when the drug has ceased to be available.

1 Being Patient-Centric, *PatientView*, August 2018

KEY FINDINGS

Why this study

Research and development is the engine that drives the pharmaceutical industry. So, not surprisingly, patients and patient groups regard innovation and its related activities as of fundamental importance to them—providing hope, a better quality of life, perhaps even a cure. It is also hardly unexpected, given a desire by the pharmaceutical industry to be more patient-centric in approach, that drug R&D activities have come under closer scrutiny by patients and patient groups. The verdict of patients and patient groups alike is that much more could be done.

The PatientView survey, *The Corporate Reputation of Pharma, 2017—from the Patient Perspective* (published in April 2018), found that 59% of its 1,300 respondent patient groups worldwide thought pharma just "Fair" to "Poor" at engaging patients/patient groups in product research; 60% of the respondent patient groups thought the same for pharma's record of engaging patients/patient groups in product development.

As mentioned in the Introduction to this *Being Patient-Centric* supplement, the subject of patient engagement in drug R&D is now being addressed by healthcare stakeholders other than the pharmaceutical industry, including regulators—notably the Food and Drug Administration (FDA), through its Patient-Focused Drug Development (PFDD) initiative. Despite these efforts, uncertainty nonetheless exists as to how patient-centric R&D should proceed. This supplement is therefore intended to help share with companies what patient groups believe is happening in the field of patient engagement in R&D; plus their views on where the gaps and uncertainty lie; and, critically, patient groups' opinions on where pharma should plan future interventions and patient engagement in what is, after all, the core function of the industry.

The analysis behind this *Being Patient-Centric* supplement

PatientView has been collecting the views and commentaries of patient groups on the subject of patient engagement in drug R&D since 2017, as well as obtaining feedback on these groups' self-declared proficiencies and needs in the area. PatientView has assembled written input on the subject from more than 1,500 patient groups.

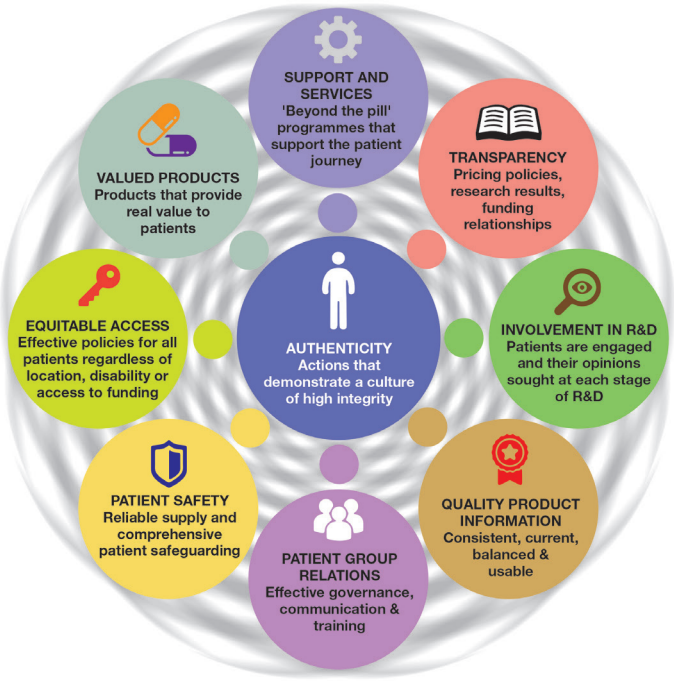
The first *Being Patient-Centric* toolkit, published in November 2017, gave an overview of the concepts of effective corporate patient-centricity (including in the area of R&D), as assessed by patient groups. This December 2018 *Being Patient-Centric* supplement, together with PatientView's August-October 2018 US survey of patient groups on the FDA's PFDD process (results published in early November 2018), seeks to provide a more detailed and thorough review of the current status and thoughts of patient groups worldwide on the entire drug R&D process, throughout the product life cycle.

This *Being Patient-Centric* supplement follows the November 2017 *Being Patient-Centric* toolkit's policy of ordering the contributions of patient groups into key themes (entitled 'fundamentals'). Each fundamental is defined by a series of questions framed to enable companies to self-evaluate the patient-centricity of their R&D, and plan patient-engagement strategies for the future.

- The full complement of patient groups' comments can be found in Appendices 2-4, ordered into the key fundamentals.
- The revised patient-centric R&D toolkit is to be found in Appendix 1. In it, patient-group comments have been rewritten as 128 questions posed to companies in the form of a self-evaluation toolkit.

To get the most complete view on patient-centricity, this global patient-centricity R&D supplement can be read alongside two other publications

For a panoramic view of the corporate activities required to be patient-centric—from a patient-group perspective—and the complete self-evaluation toolkit, see PatientView's November 2017 ***Being Patient-Centric*** toolkit.



For a more detailed view of US patient engagement in R&D, see PatientView's November 2018 supplement, ***How Patients in the US can be More Closely Involved in the Research and Development of Drugs***. The report provides feedback from 104 US patient groups on the FDA's Patient-Focused Drug Development (PFDD) initiative.

CASE STUDY

The Barth Syndrome Foundation's 2018 externally-led PFDD meeting

Key messages from a US national rare-disease patient group about how to run an externally-led meeting through the US Food and Drug Administration's PFDD initiative

On July 18th 2018, the New York-headquartered Barth Syndrome Foundation, and its international affiliate patient groups, ran an externally-led Patient-Focused Drug Development (PFDD) meeting. In a November 2018 interview with PatientView, Emily Milligan, Executive Director of the Barth Syndrome Foundation, and Michaela Damin, Chairperson of the UK-based Barth Syndrome Trust, share what they learned from their experience in planning, running, and following up after the meeting.

About Barth syndrome

Barth syndrome is an x-linked genetic disorder of the TAZ gene, occurring almost exclusively in males. Estimated to affect between 1-in-300,000 to 1-in-400,000 individuals worldwide, the syndrome's symptoms include: an enlarged and weakened heart; a compromised immune system; nutritional and feeding issues; muscular weakness; exercise intolerance; and/or growth delay.

Unfortunately, no approved treatments for Barth syndrome exist. However, several clinical studies currently under way to test therapies have shown early promise. The Barth Syndrome Foundation is the only volunteer organisation that specialises in Barth syndrome and has a worldwide reach. The Foundation is dedicated to saving lives through education, advances in treatment, and finding a cure for the syndrome.

PLANNING

Time the meeting strategically

Timing was a key consideration for the Barth Syndrome Foundation when scheduling its externally-led PFDD meeting. One US clinical trial relevant to Barth syndrome was in progress, and another planned for the UK. Therefore, regulatory matters emerged as a critical agenda for the Foundation.

Organise finances

To maximise attendance at the externally-led PFDD meeting, and to keep the cost of running it from becoming excessive, the Foundation adopted a pragmatic and patient-centric decision to dedicate an afternoon to the meeting during the week of its biennial international conference (which had long been in the diary).

By integrating the externally-led PFDD meeting within a pre-planned international conference, some of the meeting's costs (such as venue, accommodation, and travel) would already be budgeted-for and organised. However, many other significant expenses still had to be met, including that of the technology required for webcasting the PFDD event globally, and for live global polling. Also, a specialised facilitator, experienced in working on PFDD meetings with the FDA, had to be employed (well in advance of the meeting).

The investment in preparing and running (and even following up after) an externally-led PFDD meeting

"Many patient groups approach an externally-led PFDD meeting as a finite event. But it is a beginning, not an end. Our Foundation now has a dialogue with the FDA, because we have put Barth syndrome on the map as a disease with great unmet need. Regulators understand much better the urgency for delivering effective therapies for Barth syndrome as a direct result of the PFDD meeting."

—Emily Milligan, Executive Director, Barth Syndrome Foundation

APPENDIX 1

PATIENT-CENTRICITY IN R&D: A REVISED AND UPDATED TOOLKIT

Based on the comments from over 1,500 patient groups worldwide 2017-2018 (see appendices 2-4)

Patient-group feedback, and further guidance on how your company can become more patient-centric in your engagement with patients in drug R&D

In November 2017, PatientView published the first-ever, evidence-based, patient-centricity toolkit, *Being Patient-Centric*, based on substantial feedback received from patient groups over the course of several years. The toolkit is aimed at pharmaceutical companies that have a mandate to be patient-centric. The toolkit describes nine key attributes of patient-centricity for companies, and goes on to provide practical support in the form of a self-evaluation questionnaire that applies to each attribute.

One of the core attributes determining effective corporate patient-centricity is that "*patients are engaged, and their opinions sought at each stage of R&D*"—a topic of much discussion in 2018 [see introduction]. Since *Being Patient-Centric* was published, PatientView has continued to receive

BEING PATIENT-CENTRIC: THE NINE ATTRIBUTES THAT DEFINE A PATIENT-CENTRIC COMPANY

DETERMINING PATIENT UNMET NEEDS FROM PATIENT FEEDBACK

- Working with patient groups from the start, and throughout.
- Identifying patients' unmet priority needs.
- Identifying what is most important to patients and carers in their day-to-day experience.

FUNDAMENTAL

WORKING WITH PATIENT GROUPS FROM THE START, AND THROUGHOUT

SELF-EVALUATION QUESTIONS

- → Do you have a culture for R&D that is committed to involve patients early on in, and throughout, the development life cycle, wherever possible?
- → Does the senior leadership team (including leaders of R&D functions in, or across, all therapy areas) clearly communicate what type of patient involvement is expected, and what objectives and actions to take?
- → Does your organisation balance patients' needs within the context of its commercial strategy and regulatory restrictions?
- → Does your organisation have a clear view of the internal culture changes required to be effective in patient-centric R&D?
- → Does your organisation lead the industry in innovating and challenging its processes for patient-centric R&D?
- → Do your medical/legal/regulatory teams have an effective approach to enabling patient engagement in R&D, while managing risk within current regulation?
- → Has your medical/legal/regulatory team worked to reduce the bureaucratic burden on patient groups in your key governance, intellectual property, non-disclosure, and other management agreements—in a way that patient groups can understand, and agree upon?
- → Do you work to understand what support patients/patient groups need, so that they can play an informed and effective role in your R&D activities?
- → Do you demonstrate to the patients/patient groups involved that you value their feedback, have listened, and how their input has informed your decisions and actions?
- → Do you have a clear strategy, budget, and process for deciding with which patient groups to engage for each R&D project?
- → Do you make it as easy as possible for those patient groups with which you want to work to contribute?
- → Within regulations, do you take any steps to support patient groups to build capacity for contributing evidence and insight throughout the R&D life cycle?

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