

Patient Engagement in Orphan Drugs

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Abstracts

Why is the patient voice critical to orphan drug success?

Patient input is critical to an orphan drug's success, but how and when should you engage and what trends and drivers are shaping their involvement? From understanding the patient journey through better clinical trial design to post-launch support initiatives, patients are essential and knowledgeable partners for Pharma.

Informed by the insights of patient advocates and pharma, this valuable expert views report illustrates the exceptional value patient insights bring to the orphan drug development process.

"It's really important to engage with patients because they know more about their disease than anybody else."

Tony Hall

Co Founder of Findacure

Answering key questions:

Understanding disease: How can pharma benefit from a deeper understanding of rare diseases through the patients' experience?

Clinical trials: When should pharma engage with patients to ensure better clinical research outcomes?

Engagement – but who? How can pharma effectively engage with multiple patient advocacy groups who are often pursuing different agendas?

What do patients want? What do patients with rare diseases want from pharma and how should industry provide information and support?

Supporting value? Can patients play an active role in supporting value claims and influencing regulatory outcomes and pricing/reimbursement decisions?

International challenge: What are the communication and clinical research challenges when an orphan disease patient pool is widely-dispersed across many countries?

With this report you will be able to:

Identify the new CDx approaches and evaluate their benefits.

Understand how and where they will impact the market and review the challenges they must overcome.

Assess the activities, policies and partnerships of CDx-progressive pharma companies.

Understand how financial and reimbursement models need to change to reflect the real value of CDx.

Learn how advanced CDx can radically improve clinical trial research and outcomes.

Formulate strategies that encourage physicians to use approved CDx.

Identify the therapy areas and conditions where next generation CDx will make an impact.

Grasp the growing importance of research consortia in the identification of biomarkers.

Key Topics Explored:

Pharma must engage with patient groups – but which ones? A review of patient organisations and advocacy groups in 17 countries for Duchenne Muscular Dystrophy illustrates the communication challenges for pharma in accessing rare disease patient populations.

Several patient organisations are working with both pharma and regulators, and often bridge the gap between the two. This facilitates discussion around unmet needs and the importance of new orphan drugs for the rare disease patients.

Outreach is no longer a one-way street, with some patient groups tracking research and actively reaching out to companies that offer something promising.

The trend of patients with rare diseases having a significant impact on the market is expected to continue through funding of new research, creating scientific programmes and even developing drugs. Pharma needs to work alongside these pioneers to support and optimise their activities.

Expert Contributors

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Tom Croce, Head, Global Patient Advocacy at Shire Pharmaceuticals

Industry expert

CEO, Biotech company

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Melissa Hogan, Founder and President, Saving Case & Friends

Dena Heath, Member of the Board of Directors at Amyloidosis Research Consortium (ARC)

Luigi Longinotti, Portfolio Manager - Orphan Drugs at Recordati

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All respondents met FirstWord's stringent screening criteria, and were compensated for participating. To encourage frank and forthright responses, their names have been withheld.

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Contents

1 EXECUTIVE SUMMARY

2 RESEARCH OBJECTIVES AND METHODOLOGY

3 CONTRIBUTORS

4 THE IMPORTANCE OF PATIENT ENGAGEMENT FOR ORPHAN DRUGS

4.1 Key findings

4.2 What are orphan drugs?

4.2.1 Market overview and size

4.3 Patient engagement is seen as critically important

4.3.1.1 Engagement provides valuable insights into the patient journey

4.3.2 Companies can harness patient input to ensure the product fits the need

4.3.3 Patient groups have a vital role to play during clinical testing

4.3.4 Patient groups will advocate with regulatory authorities and reimbursement bodies

4.4 Companies will engage with patient organisations or advocacy groups

5 CURRENT PATIENT ENGAGEMENT STRATEGIES

5.1 Key findings

5.2 Patient organisations provide a significant range of insights

5.2.1 Insight into the patient experience is a main areas of focus

5.2.2 Patient organisations are involved in a number of R&D activities

5.2.3 Disease awareness is a key activity for patient groups

5.3 Methods to ensure beneficial interaction

5.4 Early engagement is seen as key

5.5 There are challenges to effective patient engagement

5.5.1 Compliance concerns are a major hurdle for effective engagement

5.5.2 The number of different patient groups can make it hard to collaborate

6 THE PATIENT ORGANISATIONS' VIEW ON ENGAGEMENT

6.1 Key findings

6.2 The patient voice has increased in volume in recent years

6.2.1 Patient Perspective on Benefits of engaging for pharma and patient

organisations

6.3 Activities Patients Are Involved In

6.3.1 Case study: Clinical trial design and recruitment

6.3.2 Facilitating the patient-pharma relationship

6.3.3 Case study: Working with the FDA to advance the rare disease cause

6.4 Early patient engagement is not always undertaken

6.5 Challenges to effective patient engagement

6.6 While generally happy, patient groups have some key outstanding needs

6.6.1 More information is desired by the patient organisations

6.6.2 Increased support for patient organisations would be valued

7 FUTURE OUTLOOK

7.1 Key findings

7.2 Patient engagement will continue to grow in importance

7.2.1 The patient voice will be more prominent

7.2.2 The impact of patient groups is expected to increase

7.3 Recommendations for patient engagement

7.3.1 Understand the whole experience

7.3.2 Think outside the box

7.3.3 Engage early and in a meaningful way

7.3.4 Transparency and respect are key

7.3.5 Demonstrate commitment

7.3.6 A mutually beneficial partnership is the ultimate aim

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