

An analysis of the impact and barriers to patient engagement from the perspective of patient associations and pharma companies

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Poster 211



1) Introduction

- Patient engagement is an emerging approach offering benefits to both patients and pharma companies^{1,2}.
- Patients wish to participate in drug development to ensure that their needs and preferences are met and remain a priority in development^{1,2}.
- Patient engagement also allows pharmaceutical companies to improve their R&D productivity towards developing medicines that are relevant to patients and also to define the value of their products earlier to stakeholders^{3,4}.
- In the clinical phase, the input of patients can be invaluable to improve protocols and informed consent, so that they are more patient focused and understandable to patients^{3,4}.
- The result of this can increase participant recruitment and retention in clinical trials, which are usually a key block to drug development^{2,3,4}.
- This novel work aimed to evaluate patient engagement from the perspectives of patient associations and pharma across three key aspects, importance (future benefit), value (tangible impact) and barriers according to each group, plus the perceived barriers that a stakeholder group has on the other group.
- **Case study:** DevelopAKUre is a patient-led consortium for Alkaptonuria, aiming to progress Nitisinone (a potential therapy from Sobi)⁵. The AKU society has the role of leading patient recruitment and incorporating the patient perspective into DevelopAKUre clinical trials, plus offering logistical support and disseminating trial results⁵. For the SONIA II trial, 140 patients (50% of the eligible adult patient population) were needed in only 9 months and thanks to the role of the AKU Society in recruitment, plus their strong relationship with patients, DevelopAKUre was able to rapidly recruit a large proportion of the patients in a short period of time⁵.

2) Research aims

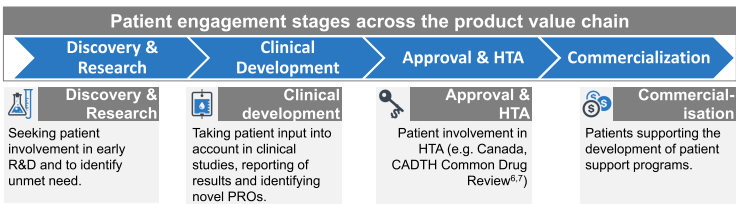
The aim of the research was to understand the three aspects below:

Importance
• Extent to which stakeholders see patient engagement as beneficial and at which stages of drug development, both now and in the future.

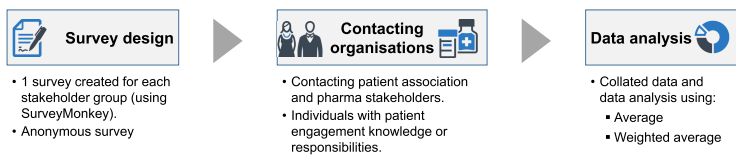
Value
• Degree to which patient engagement is seen to provide tangible impact in achieving goals in drug development.

Barriers
• Barriers inhibiting a stakeholder from performing patient engagements.
• Barriers inhibiting patient engagement that are perceived by another stakeholder group.

3) Approach



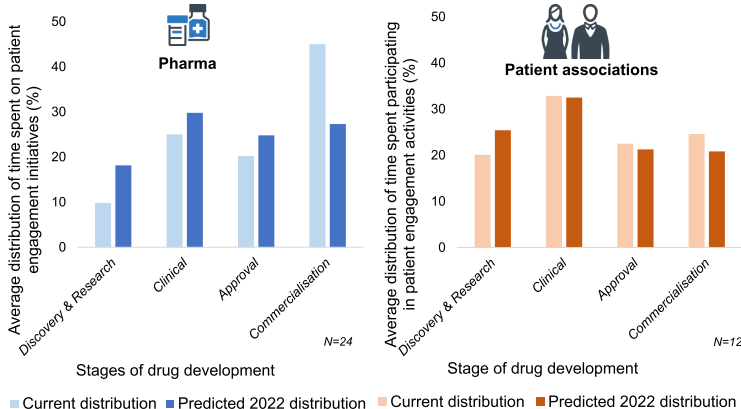
4) Methodology



Respondent disease areas	
Pharma Oncology, HIV/AIDs, Immunology, CNS, Diabetes & Cardiovascular, Gene therapy, rare diseases (gene therapy, Alzheimer's disease), Pain, Multiple Sclerosis, Respiratory, Obstetrics & Gynaecology, Haematology and generally (overarching view)	Patient associations Rare diseases (rare degenerative nerve disease, rare autoimmune disease, Cystic Fibrosis, Parkinson's disease, lysosomal storage diseases, Multiple sclerosis, overarching rare diseases), Cancer, Diabetes, Mental Health disorders, Rheumatological diseases, respiratory diseases and chronic diseases

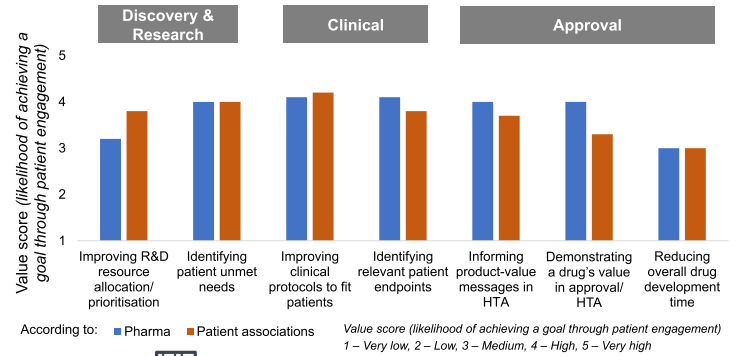
5) Importance

Current and predicted future distribution of patient engagement in drug development according to patient associations and pharma



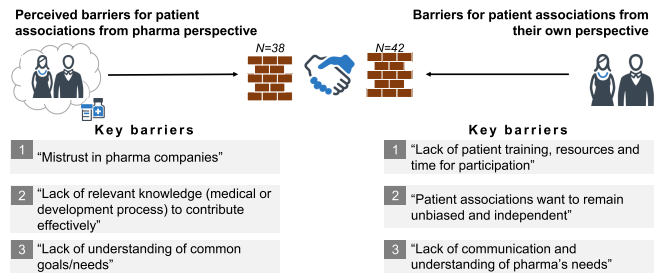
6) Value

Patient association barriers and perceived barriers of pharma from patient association perspective

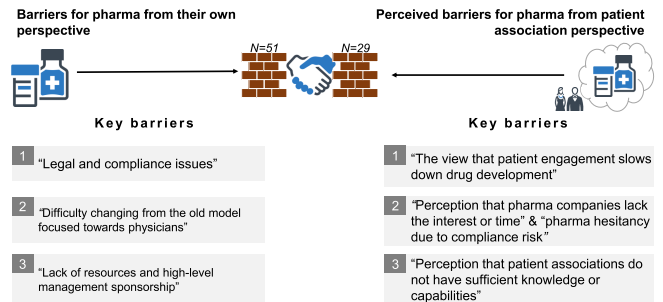


7) Barriers

Patient association barriers and the perceived barriers of pharma from the perspective of patient associations for patient engagement



Pharma barriers and the perceived barriers of patient associations from the pharma perspective for patient engagement



8) Conclusions

- This work has highlighted that both stakeholder groups believe that patient engagement will increase in importance at the earlier stages of drug development ("Discovery & Research" and "Clinical").
- Both stakeholders believe that patient engagement can provide the most tangible impact through achieving the goals in "Clinical" of improving clinical trials towards patients' needs, such as the identification of new relevant patient-reported outcomes.
- Patient associations wish to remain unbiased and independent, which is subtly different to the perception of pharma companies, who perceive patient associations to mistrust them.
- The key pharma barriers identified, compliance and a difficulty to change from the old model (clinician focused), which might appear to patient associations as a lack of interest.
- An understanding of these barriers is insightful to allow each stakeholder group to understand the view of the other and move towards solving these barriers together, thus progressing towards a paradigm of patient engagement.

9) Recommendations

- *Patient associations* should be confident in participating in patient engagement activities from their deep understanding of their conditions, which can make a real contribution, plus make use of resources such as EUPATI to develop their understanding of how their contributions can fit into the overall picture of drug development.
- *Pharma* should be more confident in collaborating with patients, who are eager to provide input, whilst patients should be considered as equal partners, who bring a valuable perspective of their condition and needs. Pharmaceutical companies should endeavour to adapt to the new paradigm of patient engagement and provide the necessary resources to allow this change to happen.
- *Both patient associations and pharma* should work together with regulatory agencies (e.g. FDA and EMA) to together build a legal and compliance framework that creates a positive environment that fosters collaboration between the two stakeholder groups.

10) Acknowledgements

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