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WHITE PAPER

END-TO-END PATIENT STRATEGY: MAKING A TREATMENT FOR PATIENTS, WITH PATIENTS

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Executive Summary

The integration of patient engagement in all stages of a product lifecycle is starting to become much more commonplace and you may start to hear the term end-to-end patient strategy used more and more often. But what does 'end-to-end' encompass, and what does this mean for drug development companies, regulators, healthcare professionals and, of course, patients themselves? Here we explore the concept and why patient voices need to be loud, and heard, during all phases of drug development.

End-to-End Patient Strategy

The need to include the patient voice at all stages of the product lifecycle is evolving. Regulators, governments and health technology assessors increasingly require patient engagement at all stages of a product life cycle. At the same time, patients are calling for more involvement. This is leading to patient engagement quickly becoming a priority market requirement.

But what do we mean by patient engagement?

> 'The effective and active collaboration of patients, patient advocates, patient representatives and/or carers in the processes and decisions within the medicines development lifecycle, along with all other relevant stakeholders when appropriate'. [1]
>
> Source: The Innovative Medicines Initiative (IMI)-funded PARADIGM (Patients Active in Research And Dialogues for an Improved Generation of Medicines) Consortium

Patient engagement is clearly a critical and important part of a medicine lifecycle, but also one where the pendulum swings in both directions. Because as well as supporting patients to be fully informed and empowered to make decisions about their health, there is also a clear opportunity for patients to support drug development and subsequent commercialisation by vocalising what they want and need from future therapies.

But what about end-to-end patient strategy specifically, and why is it important?

End-to-end patient strategy spans the entire product lifecycle, meaning that it starts at the very beginning, with drug development. According to the US Food & Drug Administration (FDA):

> 'Patient-focused drug development (PFDD) is a systematic approach to help ensure that patients' experiences, perspectives, needs, and priorities are captured and meaningfully incorporated into drug development and evaluation. As experts in what it is like to live with their condition, patients are uniquely positioned to inform the understanding of the therapeutic context for drug development and evaluation'. [2]

This end-to-end approach is seemingly one that is being readily taken up by regulating bodies and authorities, and as well as the FDA, the European Medicines Agency (EMA) and the Pharmaceuticals and Medical Devices Agency (PDMA) are demonstrating this by their recent activity and disclosure of future plans.

For example, during a period of 5 years spanning 2012 to 2017, the FDA conducted 24 disease-specific PFDD meetings and undertook numerous initiatives to bolster PFDD as part of the drug development and evaluation process.[3] Similarly, the EMA published an update to their Engagement Framework earlier this year. Notably, the objectives of the framework are to; facilitate participation of patients and consumers in regulatory activities at every stage of a medicine lifecycle; to foster methodologies to increase collection and use of patient experience data; and to ensure that patients are consulted in the development of policies and plans.[4] And the 4Fs (Firsts) strategy developed by the Pharmaceuticals and Medical Devices Agency (PDMA) has been further expanded in 2022 to account for it's first concept of 'Patient first' by promoting close collaboration with patients. [5]

There is likely much more to come, and over the next 10 years or so we can expect to see evidence of this with, for example, the FDA promoting plans for PFDD guidance documents to be published in 2025.

So, what are the benefits of implementing an end-to-end patient strategy and who will stand to gain?

The benefits of an end-to-end patient strategy are far-reaching, and patients and their carers and/or families certainly aren't the only beneficiaries. Healthcare professionals, drug development organisations and regulators also benefit from well-planned and well-executed patient engagement strategies.

For healthcare professionals, patient activation is key in supporting positive patient behaviour, and it's only logical that patient activation is a positive byproduct of patient engagement. In 2004, Dr Judith Hibbard developed the Patient Activation Measure (PAM) and in the years since, has demonstrated the positive correlation between patient activation and health outcomes. [6] Whether shared decision making (SDM) promotes patient activation or patient activation leads to patients wanting to participate more actively in SDM is not fully clear. However, as a process that respects the rights of patients to be fully involved in decisions about their care, it supports the evaluation of all available healthcare options, including patients' personal values and preferences. It allows patients and healthcare professionals to make health-related decisions together, as partners. Which, as well as supporting the patient-healthcare professional relationship, is also associated with improved patient-reported health outcomes. [7]

By being afforded the chance to be involved at an early stage of drug development, patients are able to provide a wealth of information that may help to shape clinical trials, and improve the chances of drugs being launched.[8] It's been reported that the time to bring a new pre-Phase II drug to market can be reduced by up to 2 and a half years, impacted solely by patient engagement activity. [9] Quantifiable return on investment is also demonstrable with the net present value (NPV) and expected net present value (ENVP) of a new pre-Phase II project seeing returns that are more than 500-fold that of the investment cost of patient engagement activity.[9]

Incorporating patient and public views on the benefits and risks of medical products, thereby ensuring that medicines and devices are developed in a way that considers their needs, is a key consideration for regulating bodies. [10]
We know that patients may want different things from a treatment - rapid onset of treatment versus long durable remissions, oral outpatient treatment or hospital based care, consideration of the financial impact of a treatment regime, the possible impact on future family planning and, of course, management of side effect profiles. The chance for patients and their families to voice their preferences, in addition to clinical variables will help to identify the benefit-risk (and subsequent value) of medication as part of the trial design, informing endpoints. [12,13]

> ***"A higher level of transparency on the role of Patient Engagement Data in regulatory decision making and a clear path to Patient Engagement Data-based label claims could incentivize sponsors and enable patient empowerment in treatment decisions."*** [11]

But what if our drug development is already underway? Is it too late to implement this now?

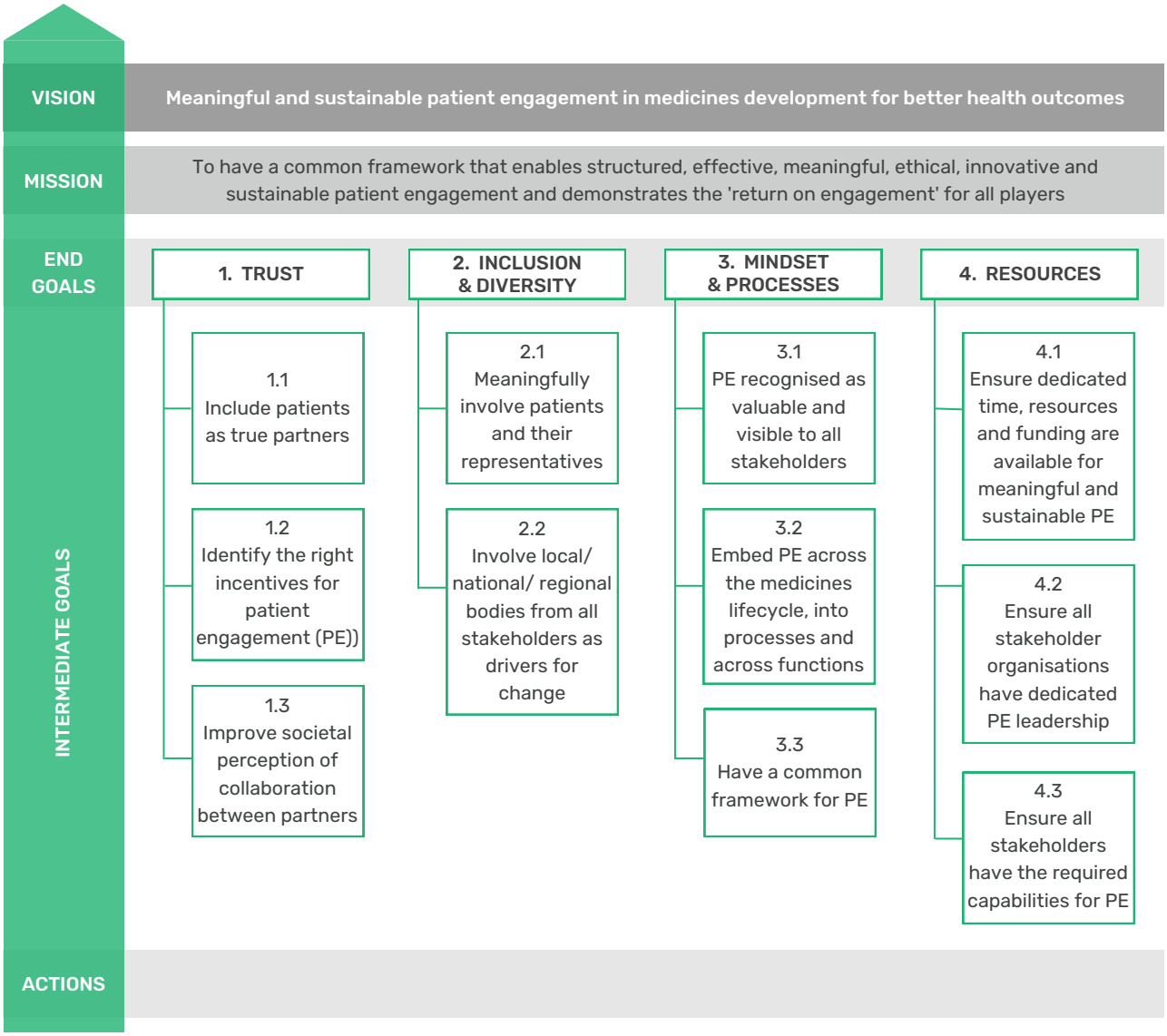
The good news is that it is never too late! The benefits of engaging patients can be seen at all stages of product development and commercialisation. So wherever you are in your product or project lifecycle, you can certainly get started today.

How and where do I start?

A good place to start is by finding out what has already been done. Some groundwork may already be in place on which you can build. Also consider the areas of your strategic imperative where patient engagement could truly make a positive impact.

By exploring the current landscape relevant to your project, you can start to identify any barriers that you may face. Obstacles to implementing patient engagement strategies can be multifactorial. They may be cultural and/or political in nature, caused by methodological issues, related to a lack of human and/or financial resources, due to a lack of knowledge and/or experience, or a result of confidentiality or conflict of interest issues. [14] Don't be afraid to dig deep into your landscape to uncover them. And be ready to face them head on. Knowing what they are, or what they may become, can help you to create a meaningful and sustainable patient engagement strategy.

There is, of course, additional thought and attention required to build your strategy. Some of this thinking has already been done thanks to PARADIGM who have published their roadmap, covering four key pillars.[15] Paying attention to these and incorporating them into your own framework may help to overcome the barriers that you will have already identified, and support you in the development of a robust and significant engagement strategy.



Source: The Innovative Medicines Initiative (IMI)-funded PARADIGM (Patients Active in Research And Dialogues for an Improved Generation of Medicines) Patient Engagement Sustainability Roadmap. [15]

Thoughts to leave you with...

Global change is already underway and yet it is clear that there is more that can, and likely will, be done in due course. For example, making it easier for underrepresented patients to be admitted to clinical trials and to be included in the development journey, will help to create a health equity that is both inclusive and diverse.[16] Likewise, more patient-centered decision making, in collaboration with patients, their carers, and their families is set to pave the way forwards. Which may beg the question – 'is it time to consider partnering with patients in the same way as you do with healthcare professionals?'. Considering that patients will likely take a more active role in deciding their treatment pathways, it may be wise to be prepared for this adjustment to the communications realm.

> ***"The integration between bench and bedside will be seamless and robust to ensure there is constant translational feedback to help tailor treatment and to inform target discovery and drug development. The success of next generation clinical trials will be based on the fundamental principles of acting locally to learn globally, and treating participants individually to advance the field collectively."*** [17]

The good news is that there are experts who can provide guidance as you navigate what may be new ways of working for you. Perhaps you need some support in implementing your designed strategy, or maybe you need an expert sounding-board who understands the fine balance that is at play here, incorporating the patient voice alongside commercial aims. If you need a helping hand to steer you in the right direction, give us a call.

Origins is passionate about working with Pharma and Biotech companies to incorporate the patient voice at all stages of product development, commercialisation and beyond. Email us now to see how we can help you to harness the power of the patient.

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