Transformational therapies demand a new mindset

Pharma is moving to a new era of radically new therapies, presenting unexpected and difficult challenges.

Such interventions take the need for robust value claims to a completely different level, with important implications for the evidence companies need to collect, and how they collect and communicate it.

Chris Gray leads Innovation and Ventures for Adelphi's Healthcare Communications agencies, whose long-running Renaissance series has been discussing the implications of 'transformational value'.

"One of the big challenges is the real need for a shift in mindset. When you're bringing a transformational therapy to market, you have to think quite differently about it. A lot of what can be achieved is unprecedented and that requires different expertise and different help," he says.

As more of these therapies emerge, there are lessons from the front-runners about the need for new approaches starting with clinical trials.

Rob Arbuckle, managing director of Adelphi Values Patient-Centered Outcomes, explains: "If the intervention is truly transformational, then part of the challenge is that existing measures may not adequately capture the new improvements you wish to demonstrate.

"There's definitely the need to think more creatively and adaptively because the claim you want to put on your label may not be as straightforward to establish as it is for more traditional therapies."

Rethinking outcomes and value evidence

There are a number of drivers behind the need to rethink value evidence and value communication. These include perceptions of transformational therapies as 'unaffordable' and the often-limited evidence available when ground-breaking treatments come to market sometimes on the basis of phase 2 data alone.

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Chris says: "One of the things that has come out a lot in the conversations through our Renaissance forums, and talking to people in medical affairs, is that there's a much greater need for more meaningful endpoints. Does the therapy meet aspirations that no-one's been able to meet before?

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"The challenge a lot of transformational therapies are hitting in getting to patients, is that HTA bodies are not necessarily set up yet to do their assessments off the back of very limited trial data," says Louise Heron, senior director, Adelphi Values PROVE. "There is still a hesitancy to say yes I can justify this, and that is a change that really does need to happen."

Changes are afoot – what to look for

These are certainly not easy issues to deal with, but there are emerging signs of innovative approaches to evidencing and communicating transformational value within pharma, regulatory and academic circles.

We are starting to see a more pragmatic approach to design of clinical trials, by for example looking at the whole patient journey. "Capturing the shift change for the patient allows economic modelling in a way which traditional frameworks are unable to achieve" explains Louise. Similarly, Rob has seen a huge growth in exit interviews:

"If it's truly novel, the trial exit interview helps to understand the value, what difference it is making to a patient's life, and in some cases has helped to convince regulators that the benefits are meaningful and important, and so tip the balance towards the product getting approved".

"There's a lot of buzz around wearables and digital, a lot of our clients are starting to include these measures in trials, but there is still work to be done to define and validate meaningful endpoints".

Digital tools such as wearables or tests of performance and functioning delivered via smartphone are providing alternative measures which complement clinician and patient-reported outcomes. For Rob this has the potential to provide novel endpoints to help convince payers and regulators, but we are not there yet.

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There is also a push from influential external academic groups such as ISPOR, encouraging assessment bodies to think differently about value, a good example being the ‘value flower’.

“This essentially brings in things like Is there a value of hope? How do you quantify that?” Louise says. She believes in a few years’ time this kind of concept will be a part of HTA evaluations, so that the broader benefit of transformational therapies can be captured.

According to Tim Holbrook, director in Adelphi Real World, finding suitable real-world data to support healthcare system decisions for transformational therapies is like looking for a needle in a haystack. However, in his recent experience, there is more willingness from companies to go further afield to find it.

“Maybe not in the sample sizes or in a country that ideally they would like, but from a payer perspective they do move the goal posts if you can make the case”.

As some governments understand the value of integrated data to inform decisions, relevant evidence can be available more readily than in the European Union where access is more restricted.

“New Zealand and Australia are good examples of proactively integrating patient data at the population level to enable future research that could include things like biomarker testing. Taiwan and Korea have a lot of data linkage potential – in the US it’s more fragmented but there are some very good sources”.

Tim also predicts a growing opportunity to shape the real-world data collected proactively for future research and evidence purposes.

“There are already examples of disease specific cohorts of patients being set up with the goal of prospectively providing the required biomarker data that is not currently being collected”.

How to adapt to transformational value’s challenges

Addressing the need for different value claims from innovative therapies requires new ways of working, with different approaches to strategic planning and an increasingly pivotal role for medical affairs.

One thing that is going to be critical, according to Louise, is true cross-functional alignment in the very early stages of clinical development.

“Have a value proposition defined very early on, but have a very clear idea of how the evidence is going to be generated to support that, so the appropriate value end points are put in place that will help access at the other end, rather than broad aspirations”.

So HEOR and market access must be involved up front, but she advocates a wider collaborative approach which also includes external perspectives from payers, policy makers and influential groups such as ISPOR. “In this space there really is the opportunity for co-creation”.

In the conversations at Renaissance, says Chris, many expressed the critical need for stronger collaboration when the potential benefit is transformational, and there was strong support for medical affairs having a central, driving role.

“We had a lot of different perspectives from people in HEOR, market access, regulatory and marketing. There was a pretty strong consensus that medical affairs sit at the centre of all of this. They’re in a key position to bring all these insights and evidence together but need to be consulted much earlier to bring together a stronger medical plan”.

To identify, demonstrate, and communicate the transformational value of your therapy, Adelphi can help you plan ahead – from expert and patient engagement, to evidence generation, through to scientific communication platforms that resonate with regulators, payers and physicians.

• Read: Adelphi’s Transformational Value: Preparing for a Decade of Radical Change white paper

About the interviewees

Rob Arbuckle is managing director of Patient-Centered Outcomes at Adelphi Values PCO, where he is responsible for providing senior, strategic input on projects focussed on the selection, development and validation of patient-reported outcomes (PRO) and other clinical outcome assessments (COA) to form trial endpoints and support product label claims and market access goals. He frequently presents on the topic at conferences and symposia and has authored numerous peer reviewed publications.

Chris Gray leads Innovation and Ventures for Adelphi’s Healthcare Communications agencies and is a member of the Adelphi Group Board. Chris has been centrally involved in the development of Adelphi’s Renaissance meeting series and has also been supporting an Adelphi initiative to explore the role of purpose in healthcare companies.

Louise Heron is senior director at Adelphi Values PROVE, where she provides senior strategic input and leads teams on PROVE projects that optimise market access for new products and treatments. She also leads Adelphi’s Health Economic offering. Louise has extensive experience in leading and conducting landscaping and literature research projects and in developing impactful health economic models and payer funding tools to communicate product value to payers.

Tim Holbrook is head of innovation at Adelphi Real World, leading its secondary data Real World Evidence team to deliver observational studies that demonstrate pharmaceutical product value. He is a specialist in ex-US data sources, RWE planning and generation using claims, administrative registries and other secondary sources of data in these regions. Tim also leads the Architect RWE planning service at Adelphi, delivering RWE planning services to clients.

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