Nine for 2023, part two: healthcare’s hard problem, the prognosis for diagnosis, and key new pharmacotherapy platforms

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16 January 2023

The first part of IQVIA EMEA Thought Leadership’s Nine for 2023 three-part series, focusing on issues that will change the direction of healthcare and the pharmaceutical industry this year, covered the macro-environment – the forces countering the historic trend to increasing globalisation, the growing fragility of healthcare systems, and the strategic choice challenge faced by pharmaceutical companies as they seek to navigate this increasingly restrictive environment.

The present article focuses on opportunity, the unmet need which lies at the heart of better healthcare provision, and two areas of growth and opportunity: Point of Care Diagnostics, and the new pharmacotherapy classes which will commercialise for the first time in 2023.

The hard problem: innovation for high prevalence, chronic diseases of ageing

Last year, IQVIA predicted that 2022 would be the crunch year for Alzheimer's therapies: it is with a sense of déjà vu that the exact same prediction is contemplated for 2023. There were developments in the past 12 months, true - Biogen/Eisai's lecanemab (now branded Leqembi) showed clear success in the Clarity AD trial and received approval by FDA on 6th January 2023. However, much remains to be resolved before a true inflection point can be called, including real world evidence, access barriers, and health system readiness. All are illustrative of a challenge broader than Alzheimer's: how to drive development and uptake of meaningful innovation in the primary care sector for the chronic, high prevalence conditions suffered by ageing populations.

Across swathes of primary care, pharmacological innovation has stalled; the last new antihypertensive class was introduced in 2007 and new antibiotics have trickled in at a dangerously slow pace. Even where there has been innovation, for example in cholesterol lowering with PCSK-9s and the siRNA treatment Leqvio, the vast majority of patients are on generic versions of medicines launched in the 1990s, and innovative medicines have not transformed the treatment paradigm. This is definitely not because all unmet need is addressed: the WHO reports that the three leading causes of death globally, and growing, are ischaemic heart disease, stroke, and chronic obstructive pulmonary disease. And all are predominantly prevented or treated with medicines that are decades old.

Ageing will only compound the challenge: high income country populations are already old, but between 2015 and 2050, the proportion of the world's population aged over 60 will nearly double, from 12% to 22%, and, by 2050, 80% of older people will be living in low- and middle-income countries. Cancers aside, most of the preventative measures and treatments for conditions suffered by these ageing populations will come through primary care as they need to be deployed at scale. Encouraging innovation is therefore imperative, but innovation will only come if innovators can make sufficient return. This is why the prognosis for actual use of Alzheimer's treatments is so critical to the "hard problem" of successful innovation for chronic, high prevalence conditions.

Alzheimer's is currently the seventh-leading cause of death globally and growing fast. Even as innovative pharmacotherapies are approved, there's still intense discussion as the basic research level on the role of beta amyloid plaques in Alzheimer's. This is linked to wider questions about the role damaged proteins and their build up plays in ageing across different tissues and organs. The research that answers these questions, and creates therapeutics as a consequence, will need substantial funding over decades to come. For that, there must be commercial incentive.

The pandemic exposed the inherent fragility of even the best funded health systems, and that fragility will increase if ways to reward the development of effective preventatives or treatments for high prevalence conditions suffered by the elderly are not successfully established. In 2023, policy makers may be making decisions on funding new Alzheimer's treatments, but more broadly they must create an environment encouraging innovation to treat and prevent high prevalence, chronic conditions of age. This requires hard, strategic choices to achieve solutions –

unprecedented multi-stakeholder collaboration, public and private, and incentives for solutions which prevent health conditions and reduce health system stress.

Diagnostic prognostic: a testing inflection?

In Vitro Diagnostics (IVDs) have been placed into the spotlight by the COVID-19 pandemic. This is especially true of self-test point-of-care (POC) diagnostics, with huge swathes of the public now aware of how to collect, read, and interpret these tests. The fundamentals are compelling: approximately 2% of healthcare expenditure is on IVDs, yet, they are involved with around 66% of clinical decisions and are critical to an improved focus on primary care-centred, patient driven, preventative healthcare.

Rapid growth is forecasted from 2023 onwards: some industry observers predict that the global POC and rapid Dx market projected to reach $75bn by 2027, up from $45bn in 2022, at a CAGR of 10.7%. Innovation will power the market. The first two diagnostics that specifically test for signs of Alzheimer's, rather than being exclusionary, were approved by the FDA in 2022 from Roche and also from Fujireibo. The approval and adoption of effective Alzheimer's therapeutics would, of course, drive the reasons to use these diagnostics and vice versa. In vitro diagnostics will become more versatile: using microfluidics in POC Dx can allow for multiple tests using a single device and multiple chips for different diseases. Innovators are looking to combine multiple techs into one platform – for example, Grail Dx's multicancer early detection platform aims for full FDA approval in 2023.

A new hope: new pharmacotherapeutic platforms become commercially viable

In pharmacotherapies, 2023 will see new technology platforms start to realise their promise. The first CRISPR therapeutic will see an FDA decision on approval in 2023 – Vertex & CRISPR Therapeutics exa-cel for sickle cell disease and transfusion-dependent beta-thalassemia, which has proven almost 100% effective 3 years post-treatment.

Gene therapies, some of which are already in market, will see a strong uptick in 2023. The European Medicine Agency-approved Biomarin's Haemophilia A gene therapeutic, Roctavian, in 2022, and it is expected to be approved by the FDA in 2023. More approvals, in larger patient population conditions will elevate the ongoing discussion of how to pay for these therapies. Germany is considering pay for performance models as viable options for gene therapies, for example, and the BeNeLuxA consortium of smaller European countries have joint Health Technology Assessment for a new gene therapy, Libmeldy.

Microbiome therapeutics will see their commercial debut in 2023, as well. Ferring squeezed a global first for the approval of its faecal microbiota therapy Rebyota for C. Difficile at the end of November 2022, but in 2023 they will be joined by Seres, which expects an FDA decision in April 2023 on its C. Difficile treatment, Ser-109.

Also notable among the many innovations which could be approved in 2023 is significant progress in Respiratory Syncytial Virus (RSV), a common respiratory

infection that can have serious consequences among the elderly and infants. This includes Pfizer and GSK in the race for the first RSV vaccine approval for over-60s, as well as a long-acting mAB approved for infants with RSV infection.

The fundamental engine of innovation for the global pharmaceutical industry continues. However, as our final article will explore, there will be a shake-out in the emerging biopharmaceutical companies from which much early innovation is generated. To thrive, pharma must ask increasingly tough questions about the viability of candidates for clinical development. Companies will also have to be more innovative outside of the R&D pipeline – in vital areas such as the competition for the attention of healthcare professionals, and commitment to carbon neutrality. These issues will be explored in the final article of this series.

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Accessed on: 01/08/2023