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Supporting patients with underserved autoimmune diseases: challenges and opportunities for innovative medicines

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Effectively addressing patient and public health needs – the role of policy

Over the past several decades, public health systems around the world have contended with a series of challenges that reflect the evolving landscape of healthcare needs. Historical and contemporary examples include the eradication of smallpox, the rise and stagnation of antibiotic discovery due to growing resistance, the need for rare disease treatments, the HIV/AIDS epidemic, the growing burden of cancer, and infectious disease outbreaks such as COVID-19 [1-3].

Each of these events has highlighted the role of effective pharmaceutical innovation in addressing public health challenges and meeting patient needs. Innovative medicines not only alleviate patient suffering associated with different conditions (e.g. adding years of life and increasing the quality of life of those years), but can also reduce healthcare costs and help working age patients regain their ability to work, resulting in benefits for patients and society more broadly.

Importantly, the research and development (R&D) and subsequent assessment, reimbursement, delivery and uptake of pharmaceutical innovation do not happen in isolation, but within the context of the policy environments in which this process occurs. It is therefore essential that policies are structured in a way to facilitate patient access to new, effective therapies, while supporting a sustainable ecosystem in which to develop and deliver them. This environment is shaped by a combination of incentive frameworks, health system readiness, and regulatory approval, health technology assessment (HTA) and pricing and reimbursement (P&R) processes – all of which are crucial for enabling research advancements and translating them into meaningful impacts for patients and society.

When policy environments fall short of sufficiently supporting R&D and patient access, it can mean that it is not feasible for certain innovative medicines (e.g., in a specific disease area or drug class) to be developed and/or brought to patients in a sustainable manner [4]. Such situations create a substantial barrier to tackling public health challenges, and can result in leaving unmet needs unaddressed and patients without valuable treatments.

It is therefore crucial to avoid these situations by ensuring that pharmaceutical innovation can be developed and delivered in a way that maximizes overall welfare. This requires supporting the sustainable development and access of effective medical treatments that can successfully meet underserved patient needs, as well as broader public health needs. To achieve this, treatments must be clinically and economically viable, and be effectively utilized once approved:

- *Clinical viability* reflects a strong understanding of disease pathophysiology and burden, the potential for treatment safety and effectiveness, and whether the treatment can address an unmet medical need [5].
- *Economic viability* involves determining whether the anticipated revenue associated with a treatment could justify the substantial costs and risks incurred through investing in its development and commercialization, while supporting long-term sustainability and affordability [5].
- *Effective utilization* of approved treatments encompasses optimal patient uptake, monitoring of drug use, engagement with patients and clinicians, and implementation of safeguards against inappropriate use.

A supportive policy environment is thus one that facilitates the development of treatments that are clinically viable, economically viable and effectively utilized, in order to maximize patient outcomes and broader societal benefits. The following two case studies further illustrate the crucial role of policy interventions in addressing public health challenges:

Case study 1: Getting it right: Orphan drug policies supporting rare disease innovation

Despite substantial patient need in rare diseases, they were historically neglected by pharmaceutical innovation, given the substantial risk and limited expected return associated with small patient populations and gaps in disease knowledge. This issue came to light after the 1962 Kefauver-Harris Amendment in the US [6]. The amendment increased the cost of R&D which, combined with the high risks and low returns, made the development of rare disease treatments an unfeasible undertaking [6].

In response to this, patient advocacy and public awareness grew, leading to the 1983 Orphan Drug Act (ODA) in the United States (US), which offered financial and regulatory incentives to stimulate treatment development for rare diseases [7]. Similarly, the European Union (EU) established its own incentive framework in 2000 with the Orphan Medicinal Products Regulation, followed by the Paediatric Regulation [8].

Orphan drugs have also benefited from country-level incentives across the EU, with specific considerations within HTA and P&R processes. In Germany, provided that a product does not exceed the €30 million threshold, orphan drugs are automatically presumed to provide an additional benefit upon marketing authorization [9]. In the United Kingdom (UK), treatments for very rare diseases can benefit from the Highly Specialised Technologies (HST) appraisal pathway, which allows for a higher cost-effectiveness threshold [10]. In Spain, orphan drugs are subject to lower mandatory discounts and are exempted from the reference price system [11], while Italy provides tax reliefs, fee reductions and exemptions from clawbacks for orphan drugs [12].

These initiatives, combined with breakthroughs in genetic research, have encouraged a major rise in orphan drug development. Before the introduction of the EU Orphan Regulation, only 8 therapies were approved in Europe [13]. Following the Regulation, the number of orphan drug approvals has increased significantly from 63 in the first decade after the regulation to 133 in the second decade [14]. This surge has led to profound improvements in health outcomes for many patients – a testament to the success of policy frameworks in incentivizing innovation.

Case study 2: Getting it wrong: The boom and bust of antibiotic discovery

In the early 20th century, bacterial infections were leading causes of death [15]. The discovery of penicillin in 1929 marked a turning point, but technical limitations prevented its clinical use. In subsequent decades, scientific advances and rising demand for treatments sparked the "Golden Era" of antibiotic discovery, driving strong R&D and commercial success [16, 17].

However, innovation began to slow due to scientific and economic challenges. Stewardship policies aiming to limit antimicrobial resistance (AMR) were introduced, restricting the uptake of antibiotics [18]. Further, new regulatory policies, such as the Kefauver-Harris Amendment in 1962 and the Hatch-Waxman Act in 1984, were implemented; these introduced stringent efficacy requirements and promoted generic competition, which increased development costs while limiting price potential [19]. These developments have diminished the economic viability of antibiotics.

Today, antibiotics are seen as unviable investments. Many manufacturers have abandoned the development of antibiotics, while others at the frontline of antibiotic R&D have ceased operations due to limited revenue potential. For example, Achaogen was founded in 2002 to develop a new antibiotic (plazomicin), which received $700 million in ‘push’ funding and was included in the World Health Organization’s Essential Medicines List; despite this, Achaogen declared bankruptcy after making less than $1 million in its first year [20].

The continued rise of AMR has led to the recognition that antibiotic innovation must be stimulated to address this significant public health challenge. Recent policy initiatives have therefore emerged, which aim to promote antibiotic innovation via financial incentives. For example, a UK pilot scheme (2019–2024) tested a subscription-style funding model with two antibiotics, Fetcroja and Zavicefta, securing three-year contracts with potential extensions [21]. Following its success, the UK permanently adopted the model, expanding it to more antibiotics. Other interventions are emerging

globally, including a novel funding model in Sweden, the PASTEUR Act in the US, and the European Commission proposal of transferable data exclusivity vouchers for novel antimicrobials [21, 22].

While these efforts mark important progress in boosting antibiotic innovation, there is more to be done. Many of these models remain limited in scale, and global alignment is lacking. Without broader supportive policy interventions, these promising initiatives risk falling short of revitalizing the antibiotics pipeline and addressing the public health challenge.

These case studies demonstrate how policy interventions can either successfully stimulate innovation, as was the case for orphan drugs, or pose challenges for innovation, as exemplified by antibiotic development. Many other disease areas continue to pose challenges to patients and health systems, and these require supportive policy solutions to ensure patient and health system needs are addressed.

The potential for innovative medicines to address underserved patient and public health needs in autoimmune disease

Autoimmune diseases represent one disease area that poses challenges for patients and public health systems. Autoimmune diseases are chronic conditions that occur when the immune system attacks healthy cells, and are characterized by a high morbidity and mortality [23, 24].

The impact of autoimmune diseases on patients is profound. Quality of life is often severely affected, due to wide-ranging symptoms such as fatigue, muscle/joint pain and cognitive fog, as well as unpredictable flare-ups [24-27]. This can cause challenges planning and maintaining daily life, and takes a toll on patients' emotional and mental wellbeing, as constant discomfort and uncertainty can lead to sadness, depression and feelings of isolation [25, 26]. Further, the complex nature of autoimmune diseases, which often involve multiple organ systems, gives rise to comorbidities and complicates diagnosis and treatment [28]. The impact of autoimmune diseases touches not only patients but also their families and caregivers; many patients are reliant on caregiver support and typically require long-term management and multidisciplinary care [29].

The burden associated with autoimmune diseases extends beyond clinical impact to a considerable socioeconomic impact. The extent of care required for patients results in high healthcare utilization for autoimmune diseases; in the US alone, direct healthcare costs exceed $100 billion annually [29].

Autoimmune diseases collectively affect many people (5–10% of the global population [30, 31]) and this prevalence has been rising steadily, with a 12.5% increase in annual prevalence worldwide in recent decades [29]. There are a few well-known autoimmune diseases such as type 1 diabetes, multiple sclerosis, rheumatoid arthritis and lupus. However, there is a large number of autoimmune diseases that are lesser-known, rare conditions, such as Guillain-Barre syndrome, chronic inflammatory demyelinating polyneuropathy and myositis [32-34]. Because of the rare nature of these less-known autoimmune diseases, the populations are not large, but the conditions are typically characterized by insufficient disease knowledge, diagnostic challenges, heterogeneous clinical presentations, R&D barriers, and limited and inconsistent therapeutic approaches [32, 35]. As a result, patients with such autoimmune diseases are often underserved, suffering symptoms that are either misdiagnosed or left undiagnosed, and a lack of treatment options. The burden of rare autoimmune diseases thus constitutes a largely silent epidemic that poses challenges for both patients and society.

While some effective treatments for autoimmune diseases do exist, they have limits. First of all, current treatments primarily focus on symptom management or rely on broad immunosuppression [36]. Immunosuppression is ineffective for certain autoimmune diseases and not all patients respond to such treatments, with some patients being refractory to treatment or experiencing recurrent disease progression [37, 38]. Prolonged use of immunosuppressants is also associated with increased morbidity and mortality [37]. Such treatments are associated with several side effects, ranging from increased infection risk, hypertension and diabetes, to psychological

disturbances, organ damage and increased risk of cancer [39, 40]. Given the substantial impact of autoimmune diseases on patients, and the limitations (and in some cases, lack) of current treatments, there is a need for innovative therapies for autoimmune diseases.

Recently, the autoimmune disease sector has seen advancements in both immune system biology understanding and drug discovery, allowing for the pinpointing of specific therapeutic targets. This has shifted autoimmune disease treatment paradigms towards targeted immune modulation, with the potential for enhanced precision and fewer side effects [41, 42]. As a result, there has been a surge in investment in autoimmune disease therapeutic drug discovery and numerous targeted immune modulation therapies are in development or have already received approval [42, 43]. Examples of such treatments include neonatal Fc receptor blockers, Bruton's tyrosine kinase inhibitors and Janus kinase inhibitors, as well as emerging advancements such as new vaccine technologies and chimeric antigen receptor T-cells [36, 39]. These developments underscore the continuing demand for new autoimmune disease treatments that leverage the most current innovation available.

A key advancement in the development of new autoimmune disease treatments is the growing focus on multi-indication medicines. Many autoimmune diseases share common molecular pathways, enabling a single therapeutic agent to treat multiple indications across a range of conditions [39, 44]. This has led to many of the targeted therapies in development being investigated for multiple indications; targeted immunotherapies launched over the past 25 years have had an average of four indications per product, with one product (adalimumab) approved for 10+ indications alone [45]. Further, among the top 10 immunology pharmaceutical companies, 36% of their inflammation and immunology pipeline therapies target multiple indications [46-56]. These multi-indication medicines can bring significant value to patients by expanding the range of available treatment options within or across disease areas. They also offer the advantage of having well-understood mechanisms of action and safety profiles, which can lead to efficiency gains in terms of development time, financial investment and pre-clinical testing [45, 57]. Multi-indication medicines thus hold great potential for effectively addressing underserved patient needs in autoimmune diseases.

Challenges for innovative medicines in autoimmune diseases

Given the promising benefits associated with innovative treatments such as multi-indication medicines, they have been commonly used in disease areas such as oncology (e.g., 34% of hematological cancer treatments launched between 2011 and 2021 were subsequently approved in multiple indications [45]). However, they have also faced several challenges that can hinder their development and patient access, and these challenges have not been fully solved. The increase in multi-indication medicines in disease areas like autoimmune diseases are thus expected to face similar challenges, as well as even greater complexities due to the nature of these conditions. These challenges can arise at each stage from development to delivery, influencing regulatory reviews of safety, efficacy, and quality; HTA and payer assessments of clinical and cost effectiveness; and ultimately, physicians' adoption in clinical practice.

Regulatory approval process: Multi-indication medicines face challenges when undergoing regulatory assessment, with follow-on indications being less likely than initial indications to receive priority review (12% vs. 20%) or orphan designation (23% vs. 46%) [58]. In Europe, multi-indication medicines are not able to fully benefit from expedited regulatory pathways, as the EMA restricts conditional marketing authorization exclusively to the initial indication of a new active substance, and while accelerated assessment is not formally limited to initial indications, it is seldom granted for follow-on indications [59].

HTA: Current HTA processes struggle to fully capture the value of each indication of a multi-indication medicine, which can vary significantly based on factors such as disease stage and pathway, therapeutic effect, and availability of alternative treatment options [60]. Moreover, follow-on indications of multi-indication medicines are more likely to receive negative HTA outcomes

than initial indications (24% vs. 14%), and are more likely to be subject to clinical restrictions or managed entry agreements [57]. Further, the variability in outcome measures across autoimmune disease indications, in contrast to the common endpoints used in oncology (survival, progression), may cause additional challenges for developing indication-based agreements, as well as create difficulty when comparing results across studies and treatments [61, 62].

P&R: Setting a single, uniform price for multiple indications is problematic, as it fails to adequately reflect the value of individual indications [58, 63]. Moreover, the expansion to new indications often leads to price erosion for earlier indications, which can diminish incentives and ability to develop new indications [57, 58, 63, 64]. This has been seen in France and Germany, where drug prices decline with each approved indication (e.g., 42.7% price reduction for fourth indication vs. first indication in Germany) [57]. Additionally, with European healthcare systems facing increasing budget constraints, payers are subjecting innovative medicines to greater cost scrutiny [65]. Multi-indication medicines raise particular affordability concerns, as the expanded total patient population with each new indication increases the budget impact [57, 60]. This challenge is magnified in autoimmune disease due to the chronic treatment setting, as opposed to the acute treatment setting in disease areas like oncology [66, 67]. Lower prices are often the solution; however, in cases such as rare, underserved autoimmune diseases, small patient populations may not generate sufficient volume to offset these reductions [68]. While novel pricing approaches have been proposed for multi-indication medicines, their implementation is often restricted. Many countries lack the data infrastructure to track utilization and reimbursement by indication; Italy is the only major market with this capability, with limited tracking possible in France and Belgium [65]. Further, even if countries have sophisticated data tracking capabilities (e.g., VALTERMED in Spain [65]), the systems may not be configured for tracking use by indication, and the countries may not be able or willing to absorb the administrative burden to enable this [65]. The challenges with tracking patients by indication are even more pronounced in autoimmune diseases, as different autoimmune diseases can overlap or coexist with each other, in contrast to oncology which is defined by distinct tumor types [60]. Some countries also have varying pricing approaches and budgets between regions, which can introduce further complexity and prevent broad access to multi-indication medicines [65].

Uptake: Even after innovative treatments such as multi-indication medicines are launched, barriers to patient access can persist in clinical practice. Despite the limitations of existing therapies, they are well established as the standard of care in autoimmune diseases and accepted by physicians. Additionally, the critical need for new autoimmune disease treatments is often unrecognized because of the lower mortality rate associated with autoimmune diseases relative to other diseases, which overlooks the detrimental impact of autoimmune diseases on patients' quality of life, as well as the substantial societal burden from the cost of management over a lifetime [28, 29, 69]. Moreover, given the extended period of relatively limited innovation in certain autoimmune diseases, treatment goals have remained modest in this disease area [70, 71]. Together, these factors may contribute to complacency and reluctance among physicians and patients regarding the adoption of innovative treatments, with a risk that innovation will only be used in later lines of therapy. On top of this, autoimmune diseases are diverse conditions that are treated by different specialist physicians, thus, a multi-indication medicine will face multiple innovation adoption curves for each specialty it targets, further complicating consistent uptake into clinical practice and the ability to reach patients.

If there is a lack of action to resolve the challenges facing innovative treatments, such as multi-indication medicines, for underserved autoimmune diseases, their development, access and delivery may become unsustainable. This could prevent them from fulfilling their promise in autoimmune diseases, and lead to a collective inability to address the needs of patients, caregivers and health systems alike.

To unlock the full potential of these innovative medicines for undeserved autoimmune diseases, a coordinated, comprehensive, multi-stakeholder response is required to rethink approaches to clinical development, regulatory approval, P&R and patient access. Such intentional, proactive and timely solutions are critical to ensuring that patients with autoimmune diseases can fully benefit from the important value that innovative therapies can offer.

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