Compassionate use: Providing access to much needed treatments

What happens when a patient's only treatment hope rests with a promising but not yet locally approved therapy? At Novartis, we are providing a lifeline of access to innovative medicines under review or not yet approved locally when no other treatment options are available.

Dec 21, 2022

When tackling a disease, patients are usually offered treatments that prolong or improve life. Sadly, for some patients, there comes a point where all approved therapy options are exhausted.

One solution may be enrolment in a clinical trial, allowing patients to receive promising medicines as they are being tested. When this is not available or the patient is not suited, another option is access via 'compassionate use' (CU) programs, at Novartis referred to as 'Managed Access Programs' (MAPs), which enable access to locally unlicensed medication (generally free of charge) for patients with serious or life-threatening medical conditions.

An established approach

Programs of this kind are not new, but the recent COVID-19 pandemic has placed a spotlight on the value of CU as healthcare systems sought ways to better prevent, treat and manage a previously unseen infection, by gaining rapid access to promising investigational therapies based on emerging clinical trial evidence.
As well as benefiting patients and healthcare professionals, CU provides an opportunity to collect real-world data, which can ultimately help speed medicines approval. This is especially important in rare diseases where there are often no or very few treatment options.

"The right thing to do"

Novartis receives an average of 10,000 CU requests a year, with an approval rate of around 95 percent for the reviewed requests. Paul Aliu, who heads the Global Governance Office in the cross-divisional Chief Medical Office at Novartis, puts this high approval rate down to a genuine desire to 'do the right thing'.

> There's an underpinning philosophy in our Managed Access programs where the default is to say 'yes' unless there's a justified medical, scientific or regulatory rationale not to. Having this patient-centric mindset in place and the willingness to deploy resources in this area is essential. We have a policy that addresses patient needs, that looks at all these cases on an individual basis and makes the right decision fairly, transparently, and promptly irrespective of geography,
>
> **Paul Aliu,** Head of the Global Governance Office in the cross-divisional Chief Medical Office at Novartis

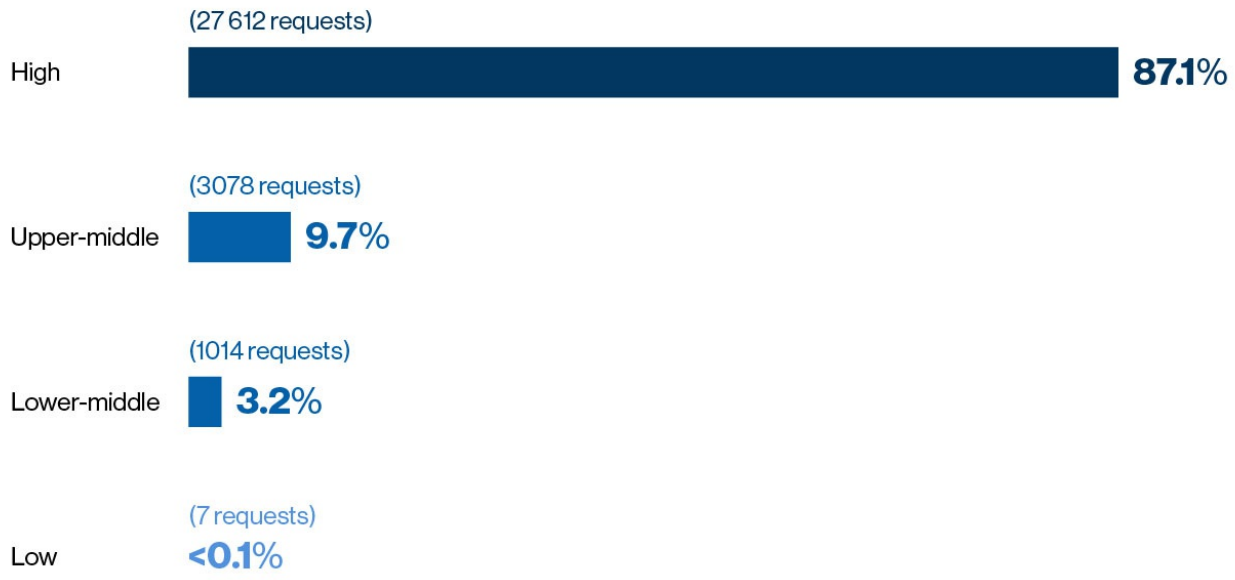
Disparity in CU access between countries

In a first of its kind study, Aliu and colleagues investigated factors associated with the 31,711 CU requests from 110 countries Novartis received over a three-year period. Although their analysis was limited to the experience of a single company and results may differ across other organizations, the hope is that their learnings can enrich the overall knowledge base in this space and translate into improved early access for patients with unmet medical needs around the world.

The study identified the following three country-level factors impacting CU requests:

1. The national level of economic development

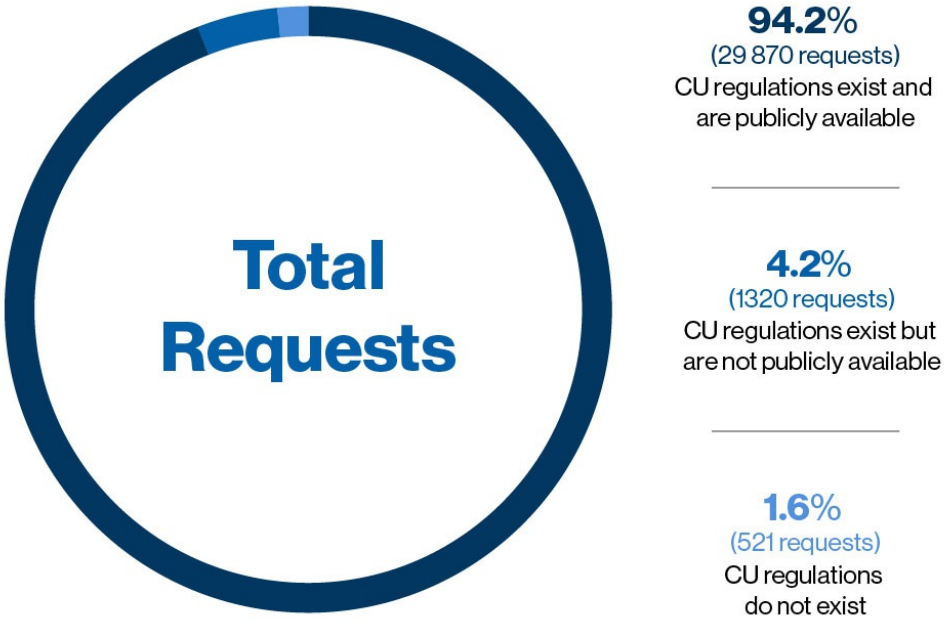
Most CU requests (87%) came from high-income countries – measured per their gross national income (GNI) per capita.



Graph 1: CU requests based on GNI per capita (Stratified Analysis by quartile)

2. The existence and public availability of CU regulations

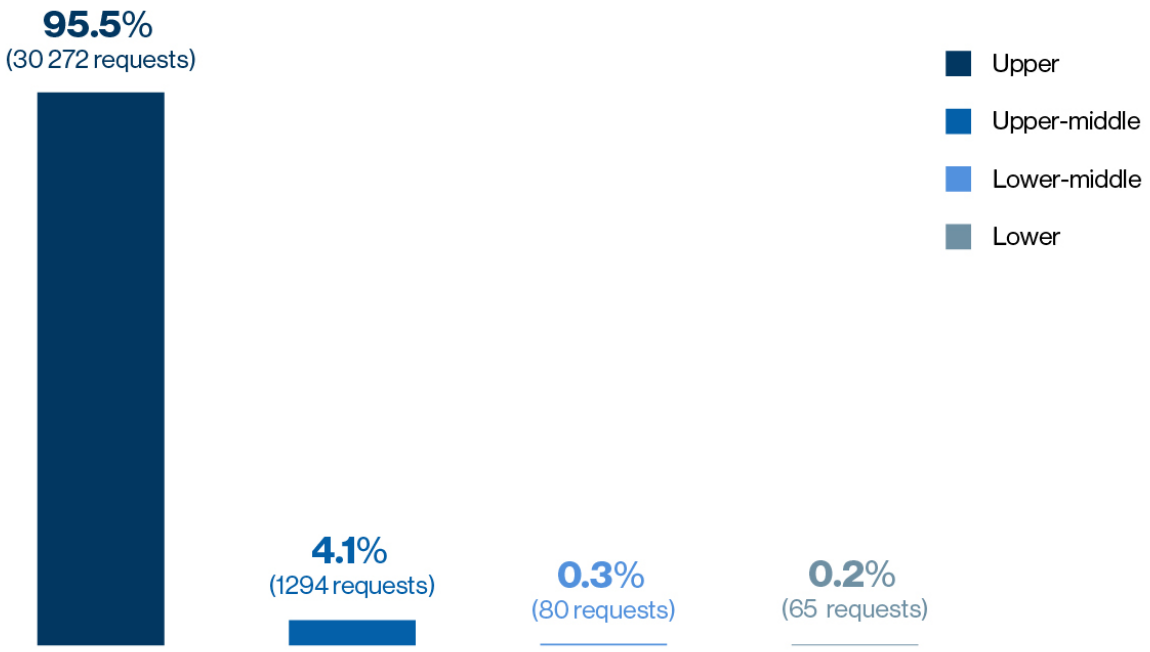
A total of 77 countries were observed to have CU regulations in place, with 54 having regulations available on their government or health authority websites. Over 94% of CU requests came from these countries where the regulations were publicly available on the internet.



Graph 2: CU requests based on existence and public availability of CU regulations

3. The number of in-country clinical trials registered on ClinicalTrials.gov

The data showed that more CU requests (95.5%) were likely to come from countries with high clinical trial activity.



Graph 3: CU requests from countries based on in-country clinical trial activity (Stratified Analysis by quartile)

Just the beginning

While these data reveal vast disparities, they also shine a light on the path forward. Aliu and team are using the data to determine the main barriers or catalysts for CU applications and how their implementation could be improved.

Novartis is collaborating with other key industry members – governments, academics, patient groups and healthcare systems in different countries – to ensure CU can help many more patients at a time when they need it most. We are committed to extending access and will continue to collaborate with various stakeholders to enhance best practices, such as the recent publication of a regulatory framework for compassionate use regulations to support the requesting physicians and other involved Healthcare Professionals, their patients, as well as Health Authorities and pharmaceutical companies.

Managed Access Programs offer us a mechanism to provide our novel innovative therapies in a responsible, ethical, speedy and safe manner to eligible patients with no other treatment options, especially when there is no time to lose and enrolment in a clinical trial is not possible. It's a true privilege to be able to address unmet patient needs in this area, and I'm proud of our teams who are constantly seeking new and compliant ways to further improve the design and execution of this important access approach.

Shreeram Aradhye, President, Global Drug Development and Chief Medical Officer for Novartis

Access at Novartis

Through our core business – the discovery, development and marketing of innovative treatments – we have helped prevent and treat diseases, ease suffering and improve quality of life for people worldwide.

But as the size and complexity of the world's healthcare challenges grow, we must widen our scope, extending our impact even further by asking: How can we effectively address the needs of underserved populations? How can we bring the benefits of our medicines to more people? And how can we do this in a way that is sustainable for our business?

Learn more

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