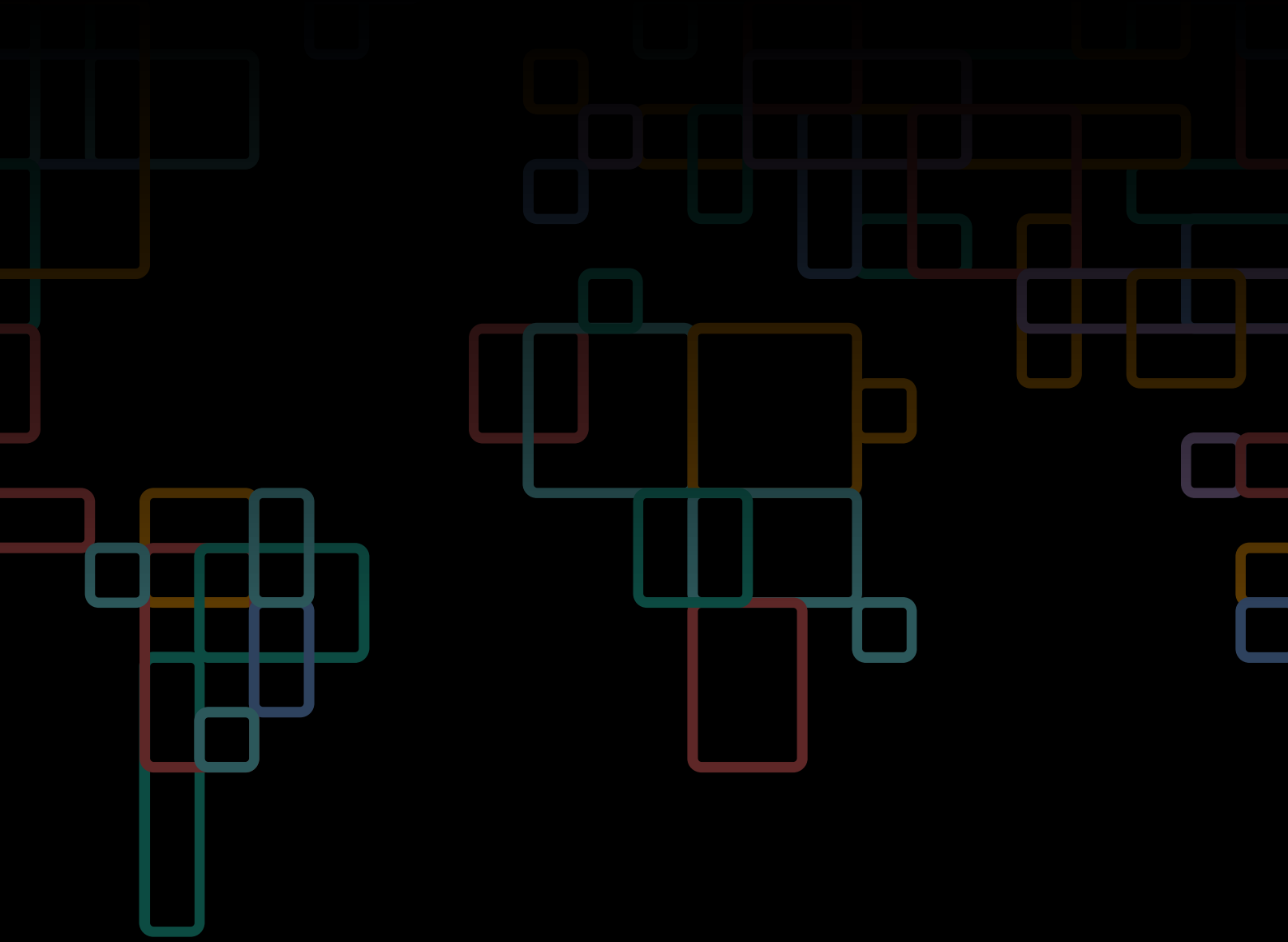
Introducing the DOLON *Institute*



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Dolon is pleased to announce the launch of the Dolon Institute, which aspires to contribute to the policy discussion that surrounds the development and availability of innovative medicines for rare and severe diseases globally. We hope to do our part to ensure that orphan medicines continue to be developed and to reach all patients who need them.

Why launch the Dolon Institute?

Debates around orphan medicine prices, patient access, innovativeness, health system sustainability, and manufacturer profits have become increasingly tense. Headlines decry the costs of novel therapies, pharmaceutical executives are scrutinised in congressional hearings, and US and EU policymakers have diminished or are considering diminishing orphan incentives. Meanwhile, the coronavirus pandemic has reinforced the importance of a dynamic pharmaceutical sector, at a time when many industry leaders feel that the innovation model is at risk.

We believe that forging a sustainable consensus amongst stakeholders remains possible. Doing so requires careful consideration of the nature of the biopharmaceutical innovation model within the constraints of political and healthcare system needs. Through the Dolon Institute, our intention is to generate new ideas, undertake rigorous analyses, and ultimately contribute to enabling honest and constructive discussions.

What will the Dolon Institute do?

We hope to meaningfully engage in the debate on medicines for rare and severe diseases by sharing evidence-based analyses and fresh, incisive perspectives. More practically, the Dolon Institute will deliver fair-handed, thought-provoking materials, including (but not limited to) peer-reviewed articles and white papers.

For patients to access orphan medicines, we see two key dimensions that should be considered.

Societal consent	Health technology assessment and pricing and reimbursement frameworks
It is well-recognised that the development of orphan medicines requires explicit, dedicated economic incentives to counterbalance the inherent challenges of their development (not least the smallness of patient populations). For these incentives to be supported, the public, and therefore politicians and policymakers, must agree that spending on pharmaceuticals for rare and severe diseases is important and just. To reach this agreement, the public, politicians and policymakers must believe in the fairness of the biopharmaceutical business model, of incentives and of the prices of orphan medicines. Currently, there is a gap between payers and policymakers, who believe industry to be (overly) profitable, and industry, who are worried that decreasing returns on research and development and increased tightening of incentives and prices will limit their ability to sustain innovation.	Patient access to medicines and price levels are governed by rigorous technical assessments of value. The design of value assessment systems, evidence requirements, acceptance of innovative contracting, etc., must be continuously refined to ensure that frameworks are tailored to the specificities of rare and severe diseases, as well as to the nature of rapid innovation.
• The biopharmaceutical innovation model • Incentives and profitability • Affordability and sustainability • Economic impact of innovation	• Value assessment and specialised rare disease pathways • Pricing approaches • Patient involvement in P&R processes

The Dolon Institute will concentrate on these two dimensions. Our primary focus for 2023-2024 will sit within the 'societal consent' dimension and will be on appraising concerns that orphan incentives and prices are overly generous, making orphan medicines over-compensated. As part of this initiative, we will further our 2020 work on the investment proposition currently afforded by orphan medicines (available here). We also intend to explore the promises and pitfalls of cost-plus pricing for orphan medicines.

Why is Dolon well equipped to tackle these topics?

From years of experience in engaging with policy developments and supporting biopharmaceutical companies with the launch of orphan medicines, Dolon has a strong understanding of the dynamics of investment, innovation and patient access, and the criticisms of them. We have built a significant portfolio of research in collaboration with biopharmaceutical companies, patient organisations, trade associations, payers, policymakers, and leading academics, helping us to grasp the perspectives of most key stakeholders. For example, we have:

- Conducted a quantitative analysis of the balance of incentives for investment in orphan medicines and the impact of the Orphan Regulation, sponsored by EFPIA (available here).
- Assessed the root causes of investment 'white spots' in extremely rare and paediatric-onset diseases and proposed a set of actions from all stakeholders that stand to meaningfully stimulate innovation in underserved areas (available here).
- Explored the economics of advanced therapy medicinal products (ATMPs) in terms of innovation and price, as well as concrete challenges and solutions to patient access to ATMPs with the RARE-IMPACT consortium (available here), with EFPIA (available here) and with Novartis (available here).
- Driven the development of a joint statement between EFPIA and EURORDIS on patient access to medicines for rare diseases (available here).
- Led a collaboration of rare disease expert stakeholders (the ORPHA-VAL group) to formulate guidelines on optimal pricing and reimbursement processes for orphan medicines (available here).
- Published a set of peer-reviewed papers that examined pharmaceutical expenditure over time and forecasted its evolution, in partnership with leading academics and industry (available here, here and here).

Building on this range of experience, the creation of the Dolon Institute will allow us to formalise our involvement in policy conversations, alongside and beyond client projects.

How can we collaborate?

We are eager to produce work that tackles the most important issues in the rare and severe disease policy space. In past projects, we have particularly enjoyed fruitful collaborations with a wide range of stakeholders (manufacturers, patient associations, trade associations, academics, think tanks, policymakers, payers, etc.). We would like to continue working in partnership with all stakeholders and thus welcome your ideas and expressions of interest.

If you're interested in working with us, please reach out to institute@dolon.com