2025 themes for pricing and access success for rare disease and oncology medicines

This year, Dolon sees several themes that will impact pricing and access success for rare disease and oncology medicines

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Theme 1

Rising pricing tension (*continued*…)

Pricing is becoming ever more critical in the context of an increasingly challenging commercial environment. The economics of the industry have worsened over the last decade: development times and costs have risen, success rates and revenues have declined [Figure 1] [1,2,3]. This is compounded by a liquidity crunch, rising capital costs, and a looming patent cliff, driving more ruthless asset selection and tight budgeting, including widespread layoffs across the industry in 2024 [4]).

In this tough environment, it is especially important to safeguard returns for those assets that do successfully make it to patients, in particular through **price protection**. But this will need to happen amid unprecedented cost pressure from payers and policymakers. Poor demographics, low productivity, inflation and rearmament are squeezing healthcare budgets and driving policy reforms aimed at drug pricing. Two notable examples in important global markets include:

1. **The U.S. Inflation Reduction Act** (**IRA**), which introduced price negotiations for some assets for the first time in U.S. history
2. **The German Financial Stabilization Act** (**GKV-FinStG**), which implemented stringent pricing guardrails

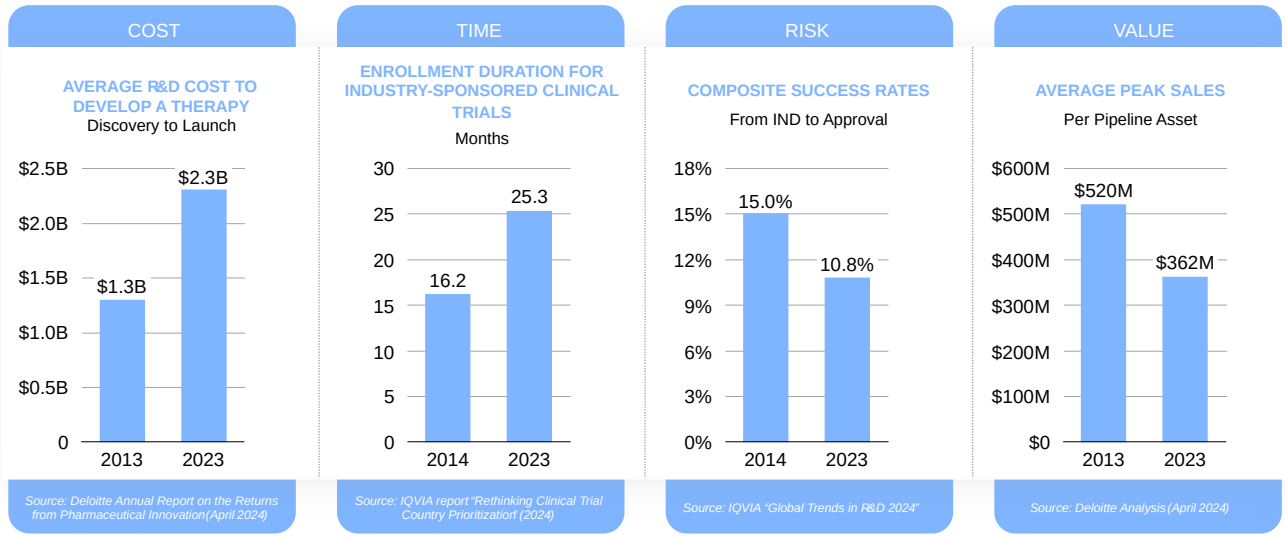
Both pieces of legislation are evolving and could see further amendments. For example, in 2025, Germany is expected to reach a final decision on constitutional complaints filed by pharmaceutical companies against the GKV-FinStG. The Trump administration has mooted repealing the IRA.

Additionally, discussions around revising ICER thresholds in cost-effectiveness-based markets gained traction in late 2024. A Lancet article proposed lowering the UK threshold to £15,000/QALY gained [5], while NICE has begun consultations on the topic [6]. Any such revisions would exacerbate existing challenges, given the threshold has remained static for two decades, effectively declining in real terms by 40% from inflation [7].

Pressure on drug pricing represents perhaps **the biggest risk for the industry in general**, especially for novel medicines targeting smaller indications, where scrutiny has been greatest.

Rising pricing tension (*continued*…)

Figure 1: Adapted from Atlas Ventures Year in Review, 2024: Analysis of Pharmaceutical business indicators over past decade [8]



What can be done?

At Dolon, we take a dual approach to supporting our clients with pricing:

- *Optimise price within payer frameworks*: Development of robust (and defensible) pricing strategies that reflect the value of medicines, but also balance considerations around sustainable innovation and patient access
- *Fight against erosion of WTP for rare and oncology innovation*: Policy engagement to explain the role of pricing in the biopharma innovation model and communicate the importance of pricing for the continued investment in orphan, oncology and highly innovative, medicine development

Theme 2

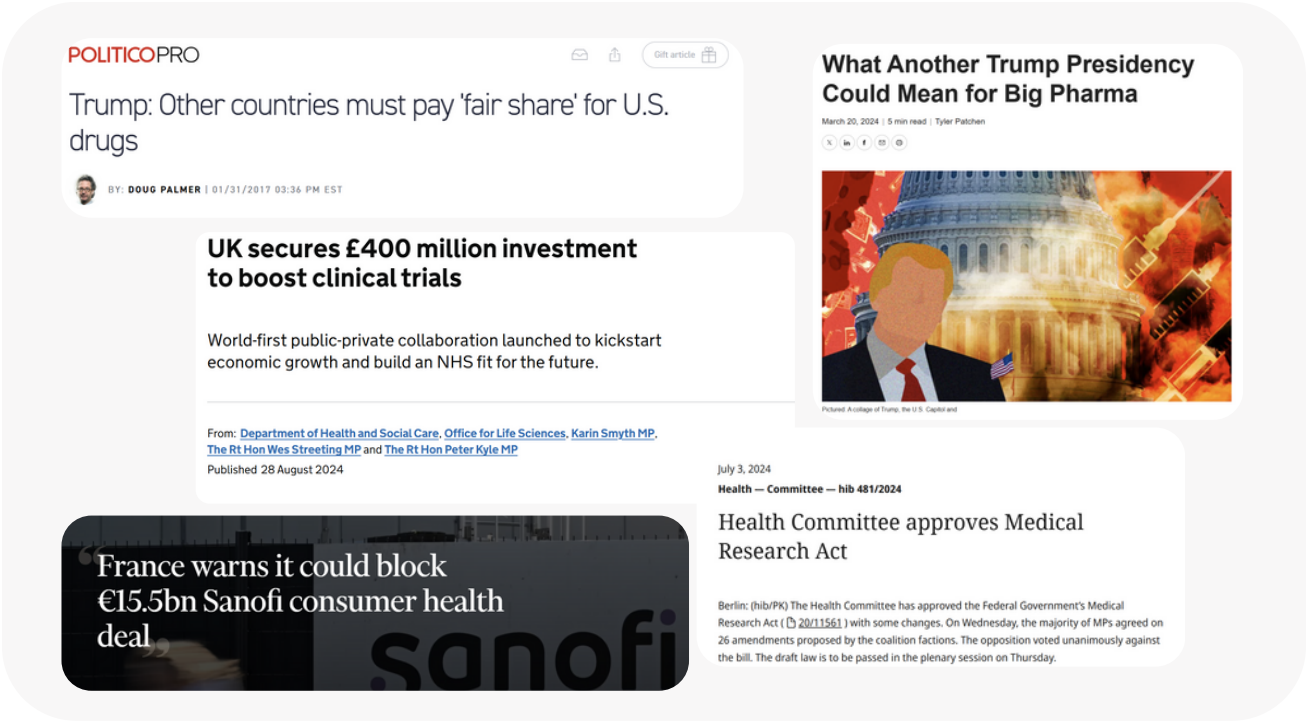
Protectionist Policies

While global scrutiny of pharmaceutical pricing practices persists, governments are increasingly adopting policies to protect their domestic life sciences sectors. **Policymakers are aiming to walk a fine line**—targeting price controls while simultaneously encouraging domestic investment, innovation and manufacturing.

Examples of recent protectionist policies and narratives include:

- **Germany**: In 2024, the German Parliament amended drug pricing and reimbursement laws under the Medical Research Act (MFG) to strengthen the country's role in medical research [9, 10]. Key measures include:
  - Price confidentiality for companies with a drug research presence in Germany conducting relevant projects
  - Incentives for clinical trials in Germany, with relaxed pricing rules for drugs involving at least 5% of participants at German sites
- **France**: Initiatives prioritise local manufacturing and reshoring critical medicines. Domestic generics production is supported via procurement clauses [11]. The government warned it could block the $15bn sale of Sanofi's consumer unit unless commitments on jobs and industrial presence are secured [12]
- **United Kingdom**: In 2024, the government launched a public-private initiative to boost clinical trial participation and modernise HTA processes for faster NHS access to treatments [13]. The Autumn Budget included measures to enhance the life sciences sector's competitiveness [14]
- **United States**: During his previous administration, President Trump proposed policies to address pricing disparities between the U.S. and Europe, a narrative that has resurfaced in his 'America First' re-election campaign [15,16]. Note: *Dolon's US country team will be publishing a note in January on the anticipated implications of Trump's election on rare and oncology medicines*

Protectionist Policies



What can be done?

In this period of heightened protectionism, companies must:

- Monitor policy trends and evaluate their implications for global and local P&R strategies
- Tailor value messaging to resonate with domestic political priorities and payer incentives
- Align pricing strategies with government sentiment, optimizing global sequencing and acceptable price differentials across markets

Theme 3

GLP-1 Wave

It's difficult to discuss important themes in the pharmaceutical business without acknowledging the transformative impact of GLP-1 therapies in obesity management (and beyond). While Dolon's primary focus is on rare diseases and oncology, we recognise that the **wave of innovation and spending in obesity medicines** will almost certainly have knock-on effects across other therapeutic areas. The GLP-1 class is forecast to exceed **$120 billion in annual sales by the next decade** [8].

This risks **displacing spending** on other therapeutic classes or indications:

- Rare disease medicines are particularly vulnerable as many payers believe that it's better to treat more patients, regardless of severity and lack of alternatives
- In rare diseases and oncology, policymakers may think that after 20 years of effort and progress it's 'job done', and there is a sense of loss of political momentum

What can be done?

Manufacturers must:

- Anticipate budgetary pressures and assess their implications for high-value therapies
- Reinforce ethical arguments for investing in rare diseases and oncology, highlighting unmet needs despite past successes
- Advocate for increased overall healthcare funding, challenging zero-sum budget assumptions

Theme 4

Oncology Crowding

Global spending on cancer medicines is expected to reach **$300 billion by 2025**, a doubling of spending from 2019 [9]. This growth has been fuelled by the explosion of new modalities and treatment approaches, including:

- **PD-(L)1 inhibitors**, **ADCs**, **CAR-T therapies** and **bispecifics**
- **Biomarker-driven treatments** and **combination therapies**

This innovation has led to **crowded treatment spaces** in key indications:

- **Non-Small Cell Lung Cancer** (**NSCLC**): Over **30 approved therapies**
- **Multiple Myeloma** (**MM**): Nearly **20 approved medicines**

This trend shows no sign of slowing, with oncology trials representing a substantial portion of all clinical trials [17].

In such a competitive environment, companies must take a holistic approach to market access strategy development in order to deliver impactful and sustainable oncology platforms.

What can be done?

Navigating increasingly complex and dynamic indications requires:

- Careful **market access strategies** to optimize positioning and differentiation
- Management of **combination pricing dynamics**
- **Pan-indication** and **above-indication strategy and communication** to enable franchise success

Theme 5

JCA "Implementation Time”

There has been a flurry of activity throughout 2024 towards the adoption of relevant Implementing Acts and guidance documents that dictate the implementation of JCA. Whilst much has been clarified in terms of EU-level processes and requirements, significant uncertainties remain:

- Countries across Europe are adapting their timelines and assessment processes (to varying extents) to incorporate the JCA report. For example, Germany appears reluctant to overhaul its approach (see here), whereas Spain is expected to base its clinical assessment on the JCA report. The main uncertainty is the extent to which the JCA findings on relative effectiveness and uncertainty will influence / determine local assessments
- Although guidance on PICO consolidation has been published, it remains unclear how requests across the 27 Member States can be consolidated in a manageable number of PICOs. This is especially a worry for some oncology or rare indications where there might be many different SoC across countries (e.g., MM)

Guidance documents do not mention specific adaptations for rare diseases / diseases where uncertainty is unavoidable. It thus seems unlikely, at this stage, that there will be flexibility in evidence requirements for rare diseases. The main risk is that the strict evidence requirements at JCA-level would lead to constrained access if individual countries do not introduce the necessary flexibility (akin to the German exemptions).

JCA "Implementation Time”

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What can be done?

For an assessment of what JCA looks like for orphan medicines and how JCA might impact the sustainability of orphan innovation and access in Europe, please see the Dolon Institute’s latest publication ‘Joint Clinical Assessment: The finger on the scale for orphan sustainability in Europe? (see here) [18].

When considering the impact on Global and Regional Value & Access team workstreams, aside from preparation around predicting and prioritising PICOs, teams must integrate JCA thinking into value strategy development, dossier development and objection handling.

Theme 6

AI and Efficiency

The past year has been marked by intense discussion about AI—understanding where the hype ends and the genuine opportunities begin.

In the market access space, we have already seen practical applications (with mixed results), including:

- **SLR development**
- **Data extraction** (e.g., HTA and analogue analysis)
- **Data visualisation**
- **Automation of value communication materials** updates

Looking forward, we are particularly excited by advancements in:

- **Predictive analytics** (e.g., anticipating payer responses or forecasting optimal price strategies)
- **Automated market access plans** (e.g., tailoring access plans for specific geographies)
- Further automation of **value communication material development and updates**

The combination of increased **efficiency** and expanded **creativity** will ensure that 2025 is an exciting and transformative year for AI in our field.

How can Dolon help?

If you are interested to hear how Dolon is implementing AI in the work we do and thinking about AI-supported services, please reach out to Richard Dutton who leads our AI taskforce and Data & Analytics team.

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References:

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