

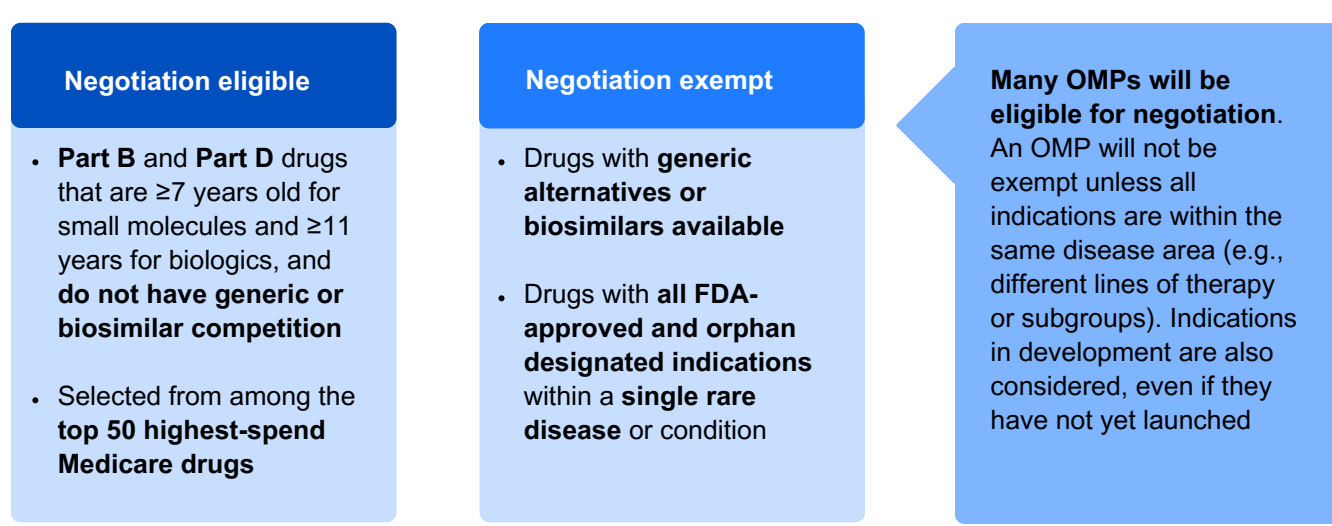
# What do the new Medicare negotiations mean for pipeline orphan therapies?

Exploring the impact of the Inflation Reduction Act on orphan drugs

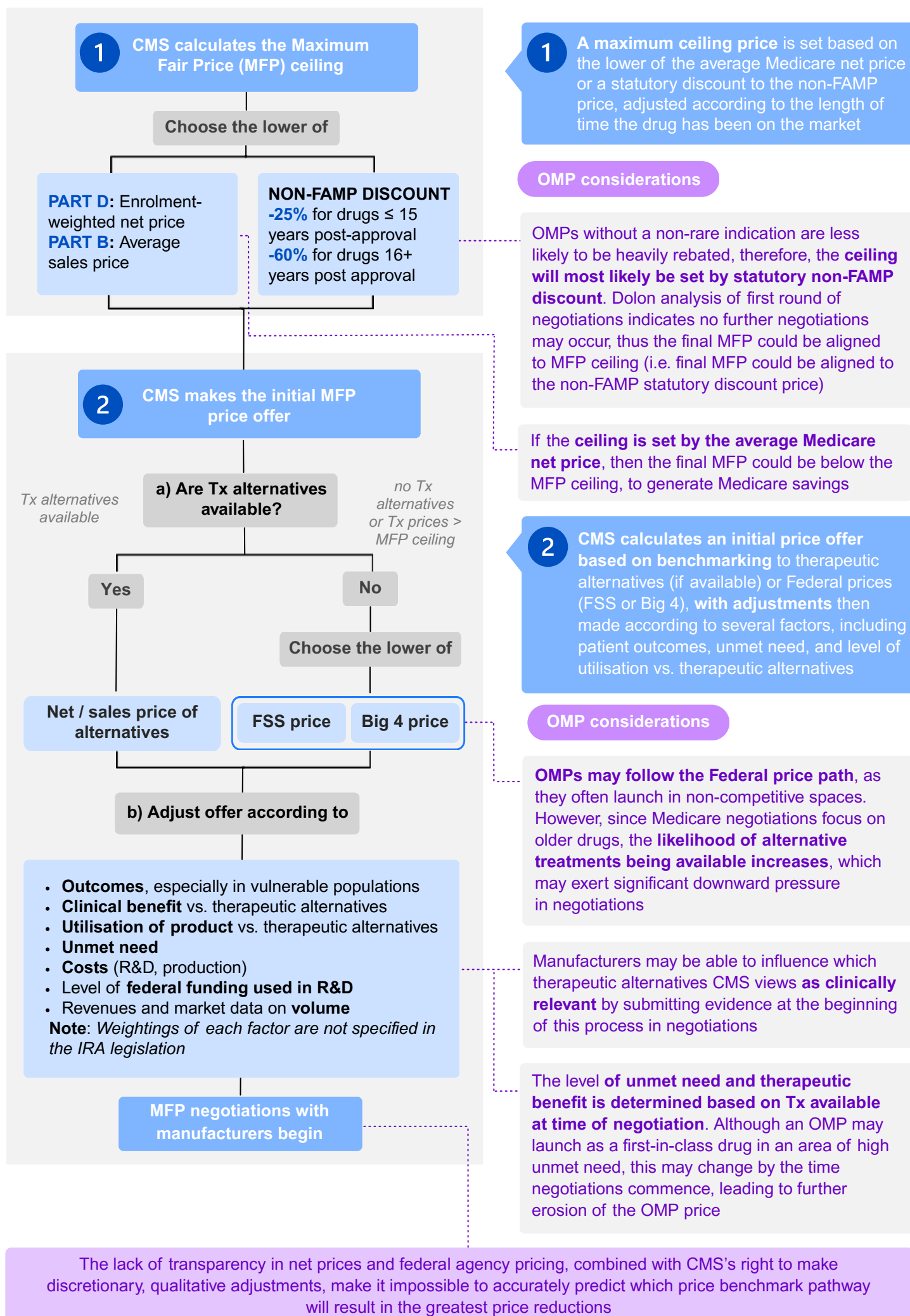
## Background

- The first round of CMS drug price negotiations led to an average cut of 63% to the list price of the selected Part D drugs, effective from 2026; in January 2025, the next 15 drugs selected for negotiations were announced, with prices effective from 2027
- The final CMS guidance<sup>1</sup> (released in October 2024) outlines the eligibility criteria for negotiations and notably allows for OMP inclusion in negotiations, under certain conditions. Although the first round of negotiations were concerned with mostly highly prevalent conditions, it is possible that OMPs could be selected for future rounds, particularly multi-indication OMPs with higher cumulative sales
- Given the more challenging clinical development and commercialization environment for OMPs, there is a risk that their eligibility for price negotiations could threaten continued rare disease drug development and indication expansion, impacting innovation and access not only in US, but also globally<sup>2</sup>
- Therefore, Dolon has conducted a holistic assessment of the CMS drug price negotiation process, to characterise the potential implications for OMPs with multiple indications that may be eligible for future negotiation

## Medicare negotiation pathway, methodology and OMP considerations



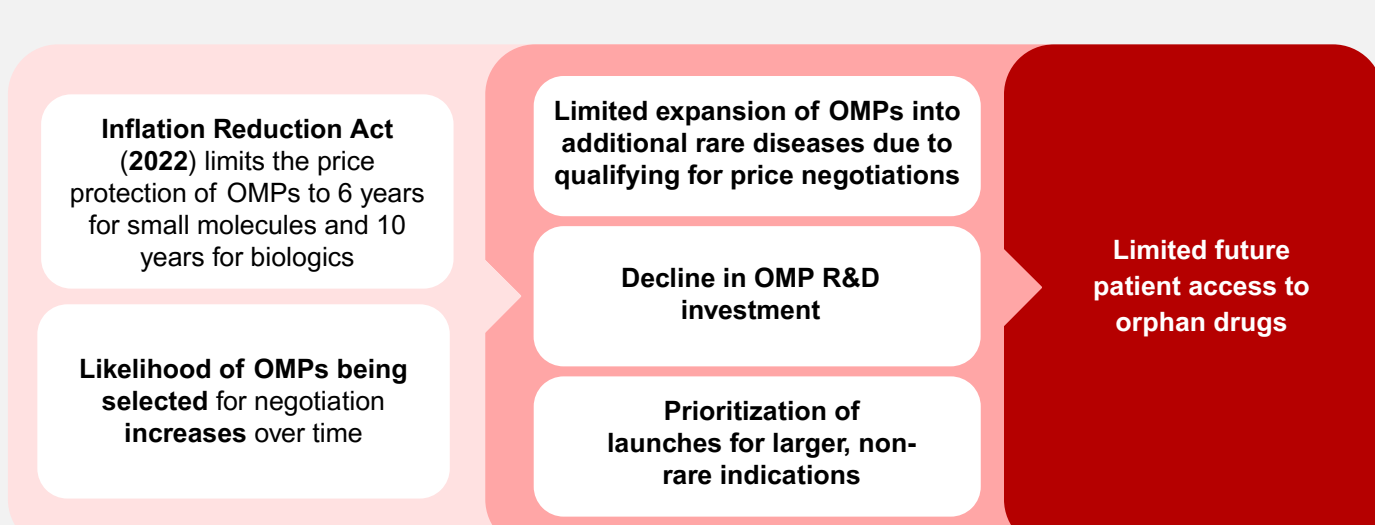
Medicare negotiations are initiated with a two-step process<sup>1</sup>:



## Forward looking implications for OMPs

- The Orphan Drug Act, which was introduced to incentivise drug development for rare diseases, provides OMPs, amongst other things, market exclusivity for seven years from FDA approval, essentially providing price protection for this period
- These incentives are crucial for continued innovation in rare diseases, given their high risk of failure, challenges in development and uncertain return on investment<sup>2,5</sup>; before the Act was passed, very few OMPs had been developed<sup>3,4</sup>
- However, the IRA effectively reduces the protection offered to OMPs under the Orphan Drug Act for small molecules. Small molecule OMPs are now eligible for negotiations in a shorter time (six years)
- Additionally, the likelihood that OMPs are selected for negotiations will increase for future negotiations, as the negotiations for drugs with high Medicare sales move towards less prevalent therapeutic areas
- These factors, alongside becoming eligible for negotiations if the OMP gains an additional indication in a separate disease or condition, will likely discourage OMP manufacturers from expanding into further rare diseases with high Medicare part B/D sales potential
- Furthermore, with the difficulty in predicting the impact of potential price negotiations, manufacturers may start to prioritise the US launch of larger, non-rare indications with larger non-Medicare components, to minimise future risk to business
  - This could place further pressure on the EU launch environment by reducing the launch of novel orphan indications
- As a result, R&D in OMPs could decline in the long-term limiting future patient access to potentially life-changing therapies

### Summary of Implications for OMPs



## What can be done?

Dolon's market access policy team can build policy engagement on IRA implications, address the decline in willingness to pay for rare disease innovation, and highlight the importance of continued investment in orphan medicine development. Dolon's pricing strategy team integrates IRA considerations into product pricing strategy for orphan medicines.

### Abbreviations

B4: Big 4 [agencies]; CMS: Centers for Medicare and Medicaid Services; FAMP: Federal average manufacturer price; FDA: Food and Drug Administration; FFS: Federal Supply Schedule; IRA: Inflation Reduction Act; MFP: Maximum Fair Price; OMP: orphan medicinal product; R&D: research and development; Tx: treatment

### References

1. CMS (2024); 2. Laurence et al. (2020); 3. FDA (2013); 4. King's Fund (2023); 5. Wong et al. (2018).