# Analysis of 20 Years of Legislative Trends to Predict Future Investment and Access to Orphan Drugs

HPR149

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## Objective

To identify legislative trends in the United States (US) that may influence investment and access to orphan drugs (ODs) and policies that can be impacted by changes in federal spending by examining the frequency, volume, and topics of rare disease (RD)/OD-related laws passed over the last 20 years.

## Background

- Government legislation profoundly shapes the US pharmaceutical industry's investment, pricing, and market access strategies for RDs and ODs. The Orphan Drug Act boosted OD availability from 38 to over 600 since its enactment in 1983,[1] enhancing market availability. The Inflation Reduction Act has resulted in many companies rethinking investments and clinical development pipelines for multi-indication drugs as the FDA have approved roughly one-quarter of ODs from 2003 to 2022 for at least 1 follow-on indication.[2,3]
- Given the number of recent changes to federal spending, it is important to understand what legislation exists surrounding RDs, as knowledge of current legislation can help stakeholders mitigate risks associated with unpredictable policy shifts.

## Methods

- An overview of the methodology is presented in **Figure 1**.
- On 3 January 2025, targeted searches of the Library of Congress were conducted to identify RD/OD-related legislation passed since 3 January 2005 searching for keywords in bill titles, summaries, and text.[4]
- Information on the presiding president, status, introduction and enactment dates, and relevance to RD community needs for each bill identified in searches were extracted into a predefined grid and qualitatively analyzed.
- RD/OD-related bills enacted as law were then categorized based on a previous framework used to review policies for rare diseases in the context of key patient needs across 11 countries.[5] As described by Dharssi (2017), the following elements were evaluated for each bill: access to treatment, diagnosis programs, coordination of care, research, and patient engagement.

## Results

### RD/OD-Related Bills Enacted as Law

- Of the 278 RD/OD-related bills introduced to Congress since 2005, 19 (7%) became law (**Figure 2**).
  - Of the 19 bills that were enacted, 13/19 (68%) were introduced from 2005–2015, compared to 6/19 (32%) from 2016–2025.
- 12/19 (63%) of RD/OD-related legislation focused on promoting RD/OD research through funding, awards for clinical trial coordination, and patient recruitment incentives.
- 9/19 (47%) RD/OD-related laws focused on patient engagement, 6/19 (32%) on coordination of care, 5/19 (26%) on diagnosis programs, and 4/19 (21%) on expanding access to ODs.

### RD/OD-Related Legislation by Presidential Term

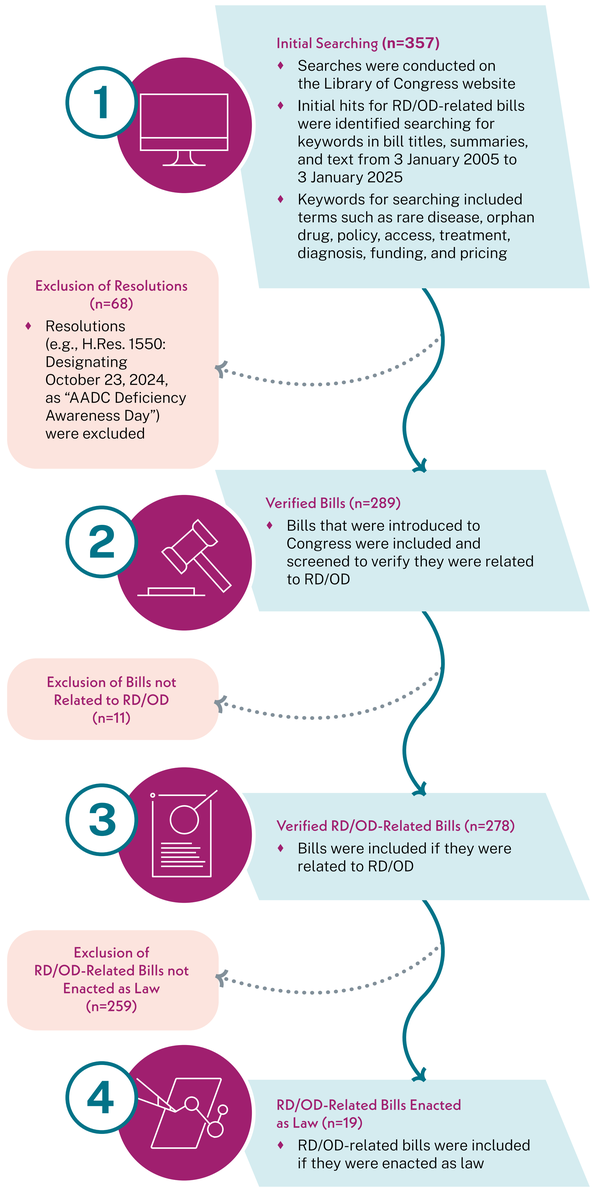
- The highest number of laws (6/19 [32%]) were passed during President Barack Obama's first term (111th and 112th Congresses) (**Figure 3**).
- 11/19 (58%) of RD/OD-related laws were passed across President Barack Obama's two terms (111th–114th Congresses).
- The lowest number of laws (2/19 [11%] each) were passed during both President Donald Trump's first term (115th and 116th Congresses) and President George W. Bush's second term (109th and 110th Congresses).

## Conclusion

Over the past 20 years, the majority of RD/OD-related legislation has been focused on promoting RD/OD research, with fewer RD/OD-related bills enacted into law from 2016 to 2025 compared with 2005–2015. While this focus may boost future OD availability, recent cuts in US government spending raise concerns about sustained policy attention. These changes in the federal budget highlight the need for increased public awareness and advocacy to ensure RD/OD issues remain a legislative priority amidst a decrease in federal spending.

## FIGURE 1

Overview of methodology




## FIGURE 2

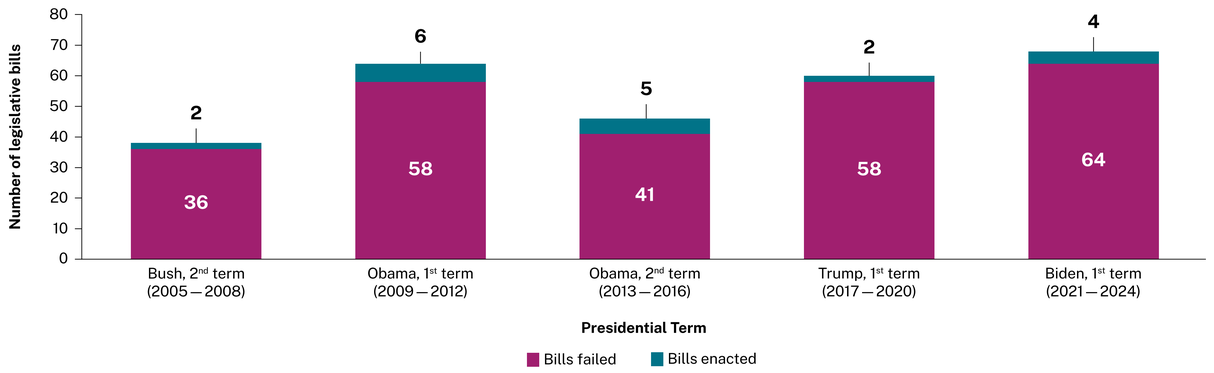
Summary of RD/OD-related legislation enacted from 3 January 2005 to 3 January 2025

| Congress | Legislation Number | Legislation Name | RD/OD-Related Policy Categories: Coordination of Care | Diagnosis Programs | Access to Treatment | Patient Engagement | Research |
|---|---|---|---|---|---|---|---|
| 109th (2005–2006) | H.R. 6164 | National Institutes of Health Reform Act of 2006 | ✗ | ✗ | ✗ | ✗ | ✓ |
| 110th (2007–2008) | H.R. 3580 | Food and Drug Administration Amendments Act of 2007 | ✗ | ✓ | ✗ | ✓ | ✓ |
| 111th (2009–2010) | H.R. 2997 | Agriculture, Rural Development, Food and Drug Administration, and Related Agencies Appropriations Act, 2010 | ✗ | ✗ | ✗ | ✗ | ✗ |
| | H.R. 3590 | Patient Protection and Affordable Care Act | ✓ | ✗ | ✗ | ✓ | ✓ |
| | H.R. 4872 | Health Care and Education Reconciliation Act of 2010 | ✓ | ✗ | ✗ | ✓ | ✗ |
| | S. 1674 | Improving Access to Clinical Trials Act of 2009 | ✗ | ✗ | ✗ | ✓ | ✓ |
| | H.R. 4994 | Medicare and Medicaid Extenders Act of 2010 | ✓ | ✗ | ✓ | ✗ | ✗ |
| 112th (2011–2012) | S. 3187 | Food and Drug Administration Safety and Innovation Act | ✗ | ✓ | ✓ | ✓ | ✗ |
| 113th (2013–2014) | S. 252 | PREEMIE Reauthorization Act | ✓ | ✓ | ✗ | ✓ | ✓ |
| | H.R. 5771 | Tax Increase Prevention Act of 2014 | ✗ | ✗ | ✗ | ✗ | ✓ |
| 114th (2015–2016) | S. 139 | Ensuring Access to Clinical Trials Act of 2015 | ✗ | ✗ | ✗ | ✓ | ✓ |
| | S. 1878 | Advancing Hope Act of 2016 | ✗ | ✓ | ✓ | ✗ | ✗ |
| | H.R. 34 | 21st Century Cures Act | ✗ | ✓ | ✓ | ✓ | ✓ |
| 115th (2017–2018) | H.R. 2430 | An act to amend the Federal Food, Drug, and Cosmetic Act† | ✗ | ✗ | ✗ | ✗ | ✓ |
| 116th (2019–2020) | H.R. 1839 | Medicaid Services Investment and Accountability Act of 2019 | ✓ | ✗ | ✗ | ✓ | ✗ |
| 117th (2021–2022) | H.R. 5376 | Inflation Reduction Act of 2022 | ✗ | ✗ | ✗ | ✗ | ✗ |
| | H.R. 6833 | Continuing Appropriations and Ukraine Supplemental Appropriations Act, 2023 | ✗ | ✗ | ✗ | ✗ | ✓ |
| | H.R. 1437 | Further Continuing Appropriations and Extensions Act, 2023 | ✗ | ✗ | ✗ | ✗ | ✓ |
| | H.R. 2617 | Consolidated Appropriations Act, 2023 | ✓ | ✗ | ✗ | ✗ | ✓ |

White/gray shading under RD/OD-Related Policy Categories corresponds to the grouping of the Congresses in the left-most column. A check mark indicates that the legislation includes RD/OD-related policy for the corresponding category, whereas an 'X' indicates that policy related to the corresponding category is not included in the legislation. †The bill's name in its entirety is 'An act to amend the Federal Food, Drug, and Cosmetic Act to revise and extend the user-fee programs for prescription drugs, medical devices, generic drugs, and biosimilar biological products, and for other purposes'.

## FIGURE 3

Characteristics of enacted RD/OD-related legislation by presidential term from 3 January 2005 to 3 January 2025




**Abbreviations: AADC:** aromatic L-amino acid decarboxylase; **H.R.:** House or Representatives; **OD:** orphan drug; **RD:** rare disease; **S.:** Senate; **US:** United States.

**References:** [1]PharmaLive.com. Recognizing the 40th Anniversary of the Orphan Drug Act. Accessed at: https://www.pharmalive.com/recognizing-the-40th-anniversary-of-the-orphan-drug-act-the-rare-disease-company-coalition-calls-on-policymakers-to-renew-commitment-to-advancing-innovation-for-rare-disease-patients/ [Last accessed 1 April 2025]; [2]The Wall Street Journal. Big Pharma Cuts R&D, Sending Shudders Through Industry. Accessed at: https://www.wsj.com/health/pharma/big-pharma-cuts-r-d-sending-shudders-through-industry-18fc4cf0 [Last accessed 1 April 2025]; [3]Chambers J.D. et al. JAMA Netw Open 2023;6:e2329006; [4]Library of Congress. Accessed at: https://www.congress.gov/browse [Last accessed 3 November 2024]; [5]Dharssi S. et al. Orphanet J Rare Dis 2017;12:63. **Acknowledgements:** The authors thank, Martina Tasende, Costello Medical, for graphic design assistance. We also thank Rachana Kilaru for their contributions.

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