

Recent Facts & Figures Related to Priority Review Vouchers' Value - 2020 to 2024

Executive Summary – As the year draws to a close, we present an updated analysis of the Priority Review Vouchers (PRV) market, which has recently experienced a notable surge in value. From 2020 to November 2024, 46 PRVs were granted throughout the three categories (37 for pediatric rare diseases, 7 for medical counter-measures, and 2 for tropical diseases), and twenty-two were traded (48% of all PRVs granted). During the same period, the aggregated PRV sales reached \$2,355 billion, with an average value of \$107 million per voucher. Notably, three recent sales by Ipsen Pharmaceuticals, Acadian Pharmaceuticals, and PTC Therapeutics have exceeded expectations, trading at approximately \$150 million or higher, a remarkable 50% premium above the average PRV value observed between 2020 and July 2024. Our analysis of PRV holding times reveals an average of 232 days between voucher grant and sale, with a range spanning from as few as 3 days to 2,656 days.

Priority Review Vouchers Granted – Table 1 summarizes the PRVs granted by the U.S. FDA from January 2020 to the end of November 2024. A total of forty-six PRVs were distributed across three distinct categories:

- 37 PRVs for pediatric rare diseases (in **Blue**)
- 7 PRVs for medical countermeasures (in **Red**)
- 2 PRVs for tropical diseases (in **Green**)

Company	Product	Year
Rhythm Pharmaceuticals	IMCIVREE	2020
Eiger Biopharmaceuticals	ZOKINVY	2020
Y-mAbs Therapeutics	DANYELZA	2020
AstraZeneca	KOSELUGO	2020
Nippon Shinyaku	VILTEPSO	2020
Vertex Pharmaceuticals	TRIKAFTA	2020
Genentech/Roche	EVRYSDI	2020
Regeneron Pharmaceuticals	INMAZEB	2020
Gilead Sciences	VEKLURY	2020
Ridgeback Biotherapeutics	EBANGA	2020
Origin Biosciences (BridgeBio Pharma)	NULIBRY	2021
BioMarin Pharmaceutical	VOXZOGO	2021
Mirum Pharmaceuticals	LIVMARLI	2021
Albireo Pharma	BILVAY	2021
Prometic Life Sciences (Liminal Biosciences)	RYPLAZIM	2021
Alnylam Pharmaceuticals	OXLUMO	2021
Enzyvant (now Sumitomo Pharma)	RETHYMIC	2021
Sarepta Therapeutics	AMONDYS 45	2021
Drug for Neglected Diseases Initiative (DNDi)	FEXINIDAZOLE	2021
Mallinckrodt (Stratatech)	STRATAGRAFT	2021
BionTech	COMIRNATY	2021

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Moderna	SPIKEVAX	2021
Bluebird bio	SKYSONA	2022
Marinus Pharmaceuticals	ZTALMY	2022
Bluebird bio	ZYNTEGLO	2022
Genzyme/Sanofi	XENPOZYME	2022
Acadia Pharmaceuticals	DAYBUE	2023
Clementia Pharmaceuticals (now Ipsen)	SOHONOS	2023
Sarepta Therapeutics	ELEVIDYS	2023
Pharming	JOENJA	2023
Reata Pharmaceuticals (now Biogen)	SKYCLARYS	2023
Amryt Pharma (now Chiesi)	FILSUVEZ	2023
Vertex Pharmaceuticals	CASGEVY	2023
Takeda Pharmaceuticals	ADZYNMA	2023
Regeneron Pharmaceuticals	VEOPOZ	2023
Krystal Biotech	VYJUVEK	2023
Chiesi	LAMZEDE	2023
Valneva	IXCHIQ	2023
Pfizer	PAXLOVID	2023
Day One Biopharmaceuticals	OJEMDA	2024
X4 Pharmaceuticals	XOLREMDI	2024
Orchard Therapeutics (now Kyowa Hakko Kirin)	LENMELDY	2024
PTC Therapeutics (former Agilis Biotherapeutics)	KEBILIDI	2024
Italfarmaco	DUVYZAT	2024
IntraBio	AQNEURSA	2024
Zevra Therapeutics	MIPLYFFA	2024

Priority Review Vouchers Sold from 2020 to November 2024 – During this period, 24 PRVs were sold according to our records, with some vouchers having been originally granted before 2020. Notable examples include the PRVs attached to Sarepta’s VYONDYS 53 (granted in 2019) and Biogen’s SPINRAZA (granted in 2016). Four other PRV owners, although they have not publicly disclosed it, have either sold or are in the process of selling their PRVs (confidential). **Table 2** below provides a comprehensive list of all sold PRVs.

Blue: pediatric rare disease – **Red:** medical countermeasure – **Green:** tropical disease

Company	Product	Year
Rhythm Pharmaceuticals	IMCIVREE	2021
Eiger Biopharmaceuticals	ZOKINVY	2020
Y-mAbs Therapeutics	DANYELZA	2020
Origin Biosciences (BridgeBio Pharma)	NULIBRY	2022
BioMarin Pharmaceutical	VOXZOGO	2022
Mirum Pharmaceuticals	LIVMARLI	2021
Albireo Pharma	BILVAY	2021
Prometic Life Sciences (Liminal Biosciences)	RYPLAZIM	2021
Sarepta Therapeutics	AMONDYS 45	2020
Mallinckrodt (Stratatech)	STRATAGRAFT	2022
Bluebird bio	SKYSONA	2023
Marinus Pharmaceuticals	ZTALMY	2022
Bluebird bio	ZYNTEGLO	2022

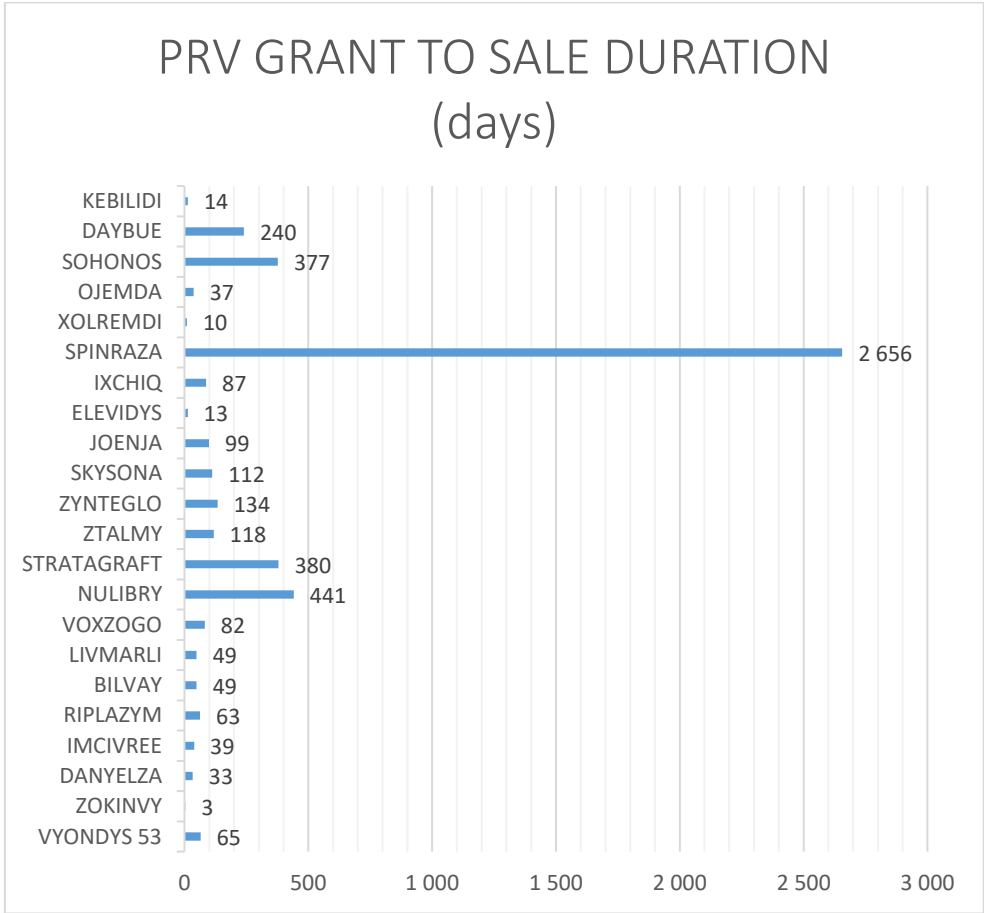
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Acadia Pharmaceuticals	DAYBUE	2024
Sarepta Therapeutics	ELEVIDYS	2023
Pharming	JOENJA	2023
Reata Pharmaceuticals (now Biogen)	SKYCLARYS	2024
Clementia Pharmaceuticals (now Ipsen)	SOHONOS	2023
Valneva	IXCHIQ	2024
Day One Biopharmaceuticals	OJEMDA	2024
X4 Pharmaceuticals	XOLREMDI	2024
PTC Therapeutics (former Agilis Biotherapeutics)	KEBILIDI	2024
Italfarmaco	DUVYZAT	2024

Time from PRV Grant to PRV Sale – During the analyzed period, the average time between a PRV grant and its sale was 232 days, with a median time of 74 days. The timeline range reveals interesting variations:

- Shortest Time: 3 days for Eiger Biopharmaceuticals' ZOKINVY (notably, the sale agreement appears to have preceded the PRV grant)
- Longest Time: 2,656 days for Biogen’s SPINRAZA

Graph 1 below illustrates the duration in days between the grant of a Priority Review Voucher (PRV) and its sale.



Legend:

- **X-axis:** Duration in days from when the PRV was granted to when it was sold.
- **Y-axis:** The specific products for which the PRVs were issued, listed vertically.
- **Bars:** The blue horizontal bars represent the number of days between the grant and sale of the PRV for each product.

PRV Value from 2020 to November 2024 – Observations on PRV values:

Historical Context:

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- From January 2000 to July 2024, PRVs traded at an average of \$100 million
- The median PRV value was \$105 million
- Typical value range:
 - Lowest standard values: \$95 million (for ZOKINVY and SKYSONA)
 - Highest standard values: \$110 million (for LIVMARLI, VOXZOGO, NULIBRY, and ZTALMY)
- Notable exception: JOENJA at \$21 million, due to pre-negotiated licensing agreement terms

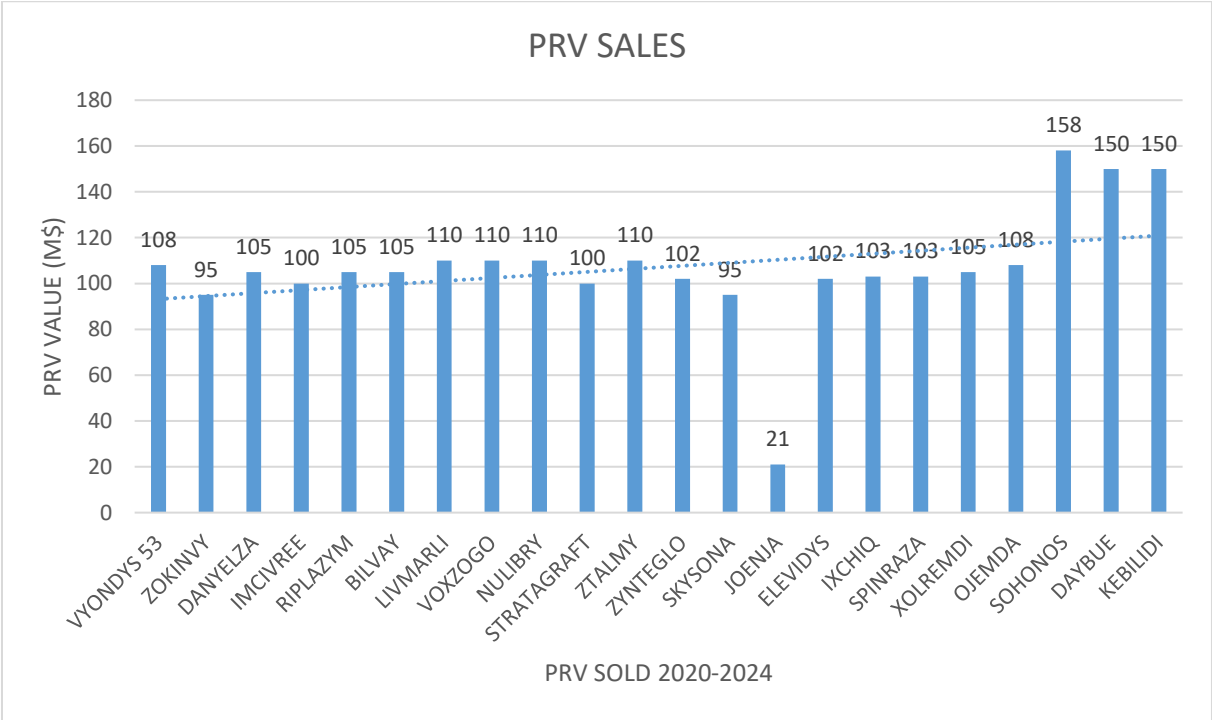
Recent Market Shift - Since August 2024, PRV values have unexpectedly increased:

- \$158 million for Ipsen Biopharmaceutical's SOHONOS-related voucher
- Two subsequent sales at \$150 million each:
 - Acadia Pharmaceuticals' DAYBUE voucher
 - PTC Therapeutics' KEBILIDI voucher

Aggregate Financial Summary:

- Total PRV sales: \$2.355 billion
- Average per company: \$118 million (across 20 PRV sellers)

Graph 2 below represents the sales of Priority Review Vouchers (PRVs) from 2020 to 2024, illustrating the value of each sale in millions of USD. Below is a proposed legend for the graph:



Legend:

- **X-axis:** Represents the PRVs sold, labeled by the products for which the vouchers were issued.
- **Y-axis:** Represents the monetary value (in millions USD) at which each PRV was sold.
- **Bars:** The individual blue bars indicate the specific sale value of each PRV.
- **Dotted Line:** A trend line that shows the general progression or average of PRV sales values over the years.

Recent Value Increase Drivers – Three interconnected factors have contributed to the dramatic increase in Priority Review Voucher (PRV) values:

- Patent Scarcity and Legislative Uncertainty
 - Limited availability of PRVs for sale

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- Potential expiration of PRV-related legislation, creating uncertainty about future renewals
- This scarcity has significantly driven up PRV values
- Company Size and PRV Monetization Strategies
 - Private and small/mid-cap companies tend to sell PRVs quickly to extend their cash runway
 - Large-cap companies typically retain PRVs for internal pipeline candidates who do not qualify for priority review
 - This divergent approach creates a unique market dynamic
- Big Pharma's Growing PRV Demand
 - Increasing interest is driven by the current and anticipated patent cliff
 - The strategic value of PRVs in managing pharmaceutical development portfolios

Limitations – Our analysis is based on 22 fully publicized PRV transactions. The study does not account for the following:

- PRV transactions that have not been publicly disclosed; and
- PRV trades where financial details were not revealed.

Methodology – This analysis relies exclusively on publicly available information regarding PRV transactions and, therefore, may not fully capture undisclosed market activities.

Conclusion – The Priority Review Voucher (PRV) market remains a dynamic force driving innovation in rare diseases, tropical diseases, and medical countermeasures. Analysis of recent trends reveals a nuanced landscape where PRV issuance, sales timelines, and valuations reflect strategic priorities and market opportunities. Smaller biotech firms, often constrained by liquidity needs, exhibit an average sale timeline of 232 days, while outliers like Biogen's SPINRAZA highlight the potential for strategic retention. Meanwhile, rising valuations, culminating in Ipsen's record-setting \$158 million sale in 2024, reflect heightened demand, legislative uncertainty, and Big Pharma's increasing reliance on PRVs as tools to hedge against patent cliffs and secure competitive market entries.

Smaller firms continue to leverage PRVs as essential financial instruments, while larger pharmaceutical companies strategically employ them to accelerate pipeline development and blockbuster launches. The reauthorization of the Rare Pediatric Disease (RPD) PRV program through 2030 is expected to sustain a robust issuance pipeline, with nine PRVs anticipated in 2024 alone. The program's ability to incentivize innovation, enhance public health preparedness, and deliver tangible value across the pharmaceutical landscape underscores its critical role in addressing underserved therapeutic areas. To sustain its transformative impact, policymakers and stakeholders must ensure the program's predictability and availability amidst evolving industry dynamics.

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With over 16 years of experience in the healthcare industry, Nana has held key roles at Pfizer and Komodo Health and co-founded Medsfinder, a healthtech platform. As CEO of Cleracs Consulting, he specializes in regulatory strategy, focusing on orphan drug regulatory affairs. Nana holds a Doctor of Pharmacy from Nova Southeastern University, a Master of Arts from IE Business School, and certificates in Health Leadership and Finance from INSEAD and Cornell University, respectively, along with a Bachelor of Pharmacy from KNUST.

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Christian is a co-founder of The PRV Fund, an initiative focused on providing non-dilutive funding to early-stage biotech companies developing treatments for rare pediatric-onset disorders. Christian has over 30 years professional background marked by his commitment to advancing rare pediatric disease drug development, from lab bench to approval. His involvement in this sector highlights his dedication to supporting innovative therapies aimed at improving the lives of children with rare diseases. He is a graduate of ESCP Europe, a European business school.

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Jean is a Partner and EVP at KYBORA, a global advisory firm specializing in M&A, licensing, fundraising, and strategic advisory services in biopharma. He contributed to the divestiture of Bayer's PRV to argenx for \$98M [1]. With over 24 years of experience, he has held key leadership roles, including CBO at Besins Healthcare and pivotal positions at Avadel Pharmaceuticals, Micromet (now Amgen), and Crucell (now J&J). He was the founding CEO of Avidis (now Osivax) and has worked with Nobel laureates during his postdoctoral research. Jean holds a PhD in Biochemistry and Molecular Biology and has led significant industry partnerships and transactions throughout his career.

[1] On November 2020, argenx enters into agreement to acquire Priority Review Voucher <https://www.globenewswire.com/news-release/2020/11/23/2131371/0/en/argenx-Enters-Into-Agreement-To-Acquire-Priority-Review-Voucher.html>.