



Orphan Drug Market - Past, Present & Future



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Understanding Orphan and Rare Diseases

Rare Disease

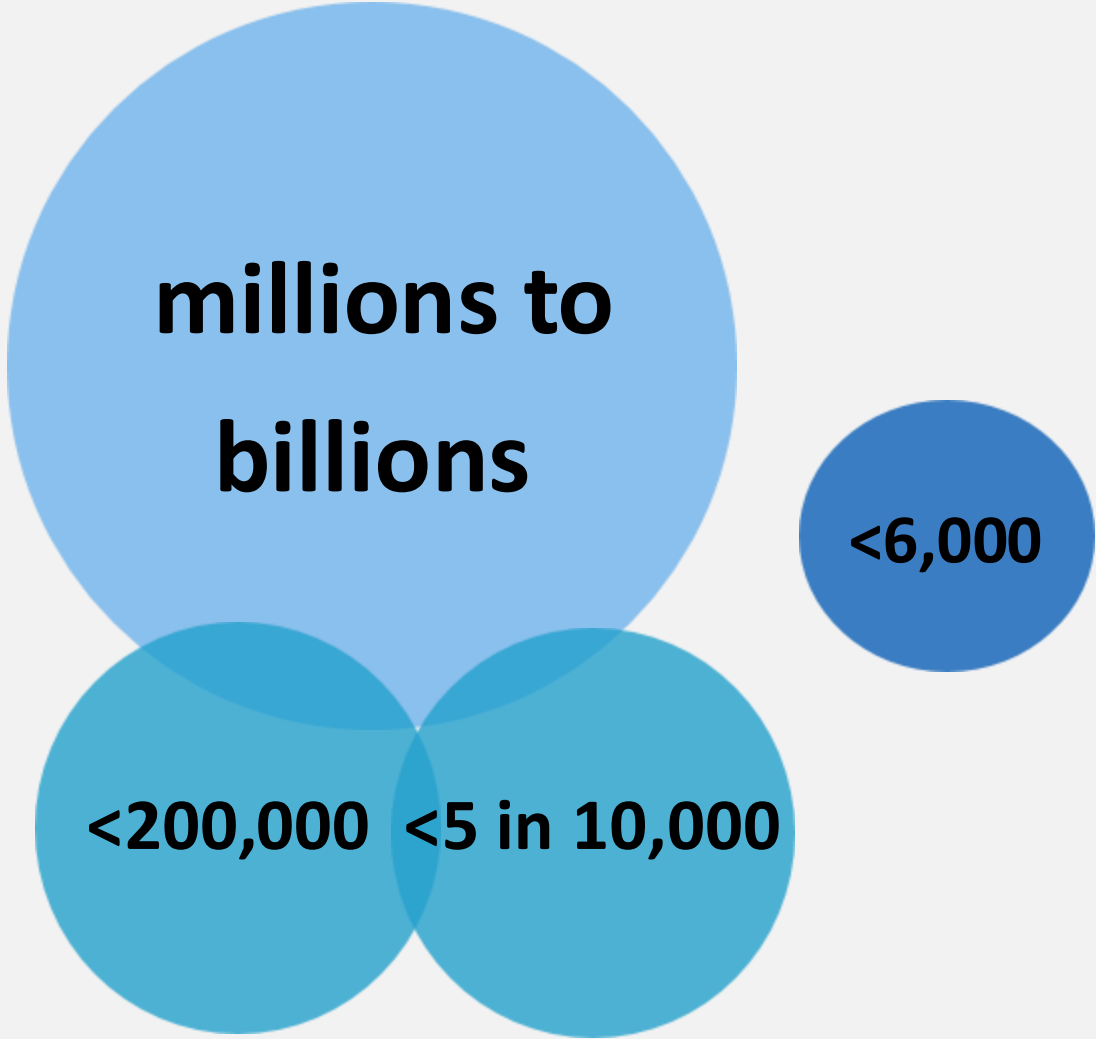
US <200,000 people nationwide.¹
EU <5 in 10,000 people in the population.²

Orphan Drug

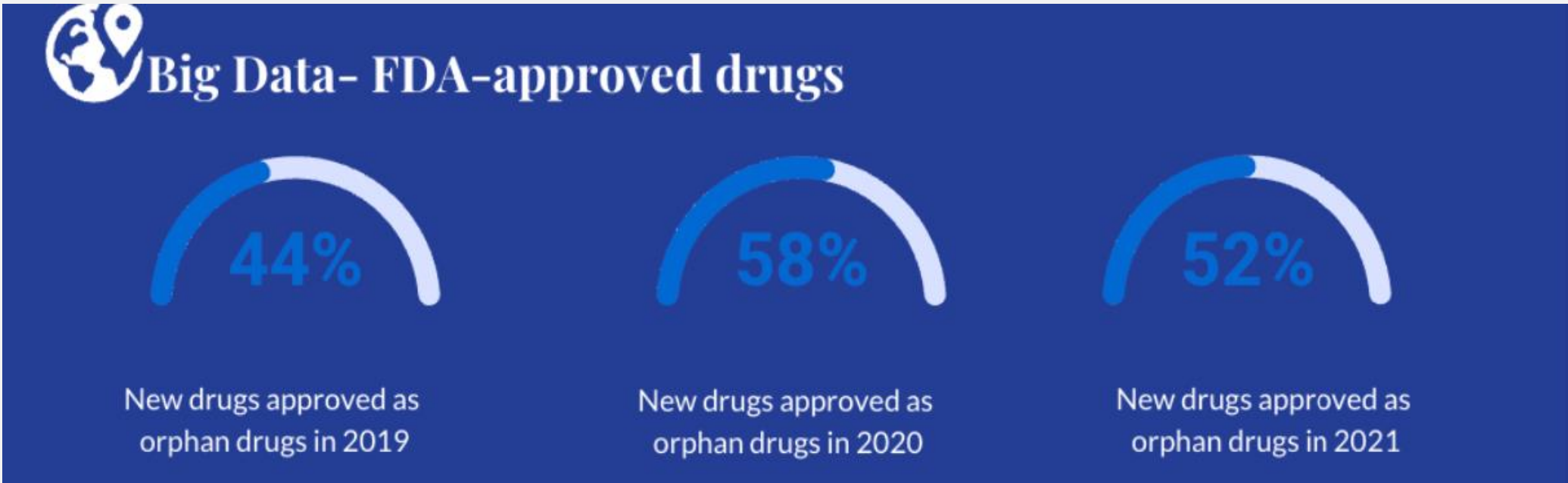
A medicine developed specifically to treat a rare disease, granted special incentives to encourage development.^{1 2}

Ultra-Orphan Drug

US fewer than 6,000 patients.
UK <1 in 50,000 people in the UK.³



Addressing severe unmet medical needs.



1. FDA – Orphan Drug Designation: Frequently Asked Questions, 2024
2.EMA – Orphan designation: Overview, 2024
3.NICE – Highly Specialized Technologies guidance for ultra-orphan drugs, 2017
4.Orphan Pharma Report, Dimitrina Dimitrova

Orphan Market Size



\$195.2 Billion¹

60% US²

2x Higher CAGR¹

1. EvaluatePharma. World Preview 2024, Outlook to 2030. 2024.

2. Grand View Research. Global Pharmaceutical Market Report 2025–2030. 2025.

Investing in Small Patient Populations

Orphan Incentives



Reaching Patients Sooner

Time to market ~5 yrs (vs. 10–12)
Patient & societal support speeds access¹.



Market Exclusivity

Long market exclusivity (7–10 years US/EU)
Strong payer acceptance for severe disease².



Additional FDA Support

Dedicated Orphan Products Unit
FastTrack, Priority Review, waived fees³.



Legislative Wins

Orphan Drug Act (1983)
2025 Bill expanded protections: pricing exemptions from Medicare negotiations providers⁴.

1. EvaluatePharma. World Preview 2024, Outlook to 2030. 2024.

2.FDA & EMA – Orphan designation: Overview, 2024.

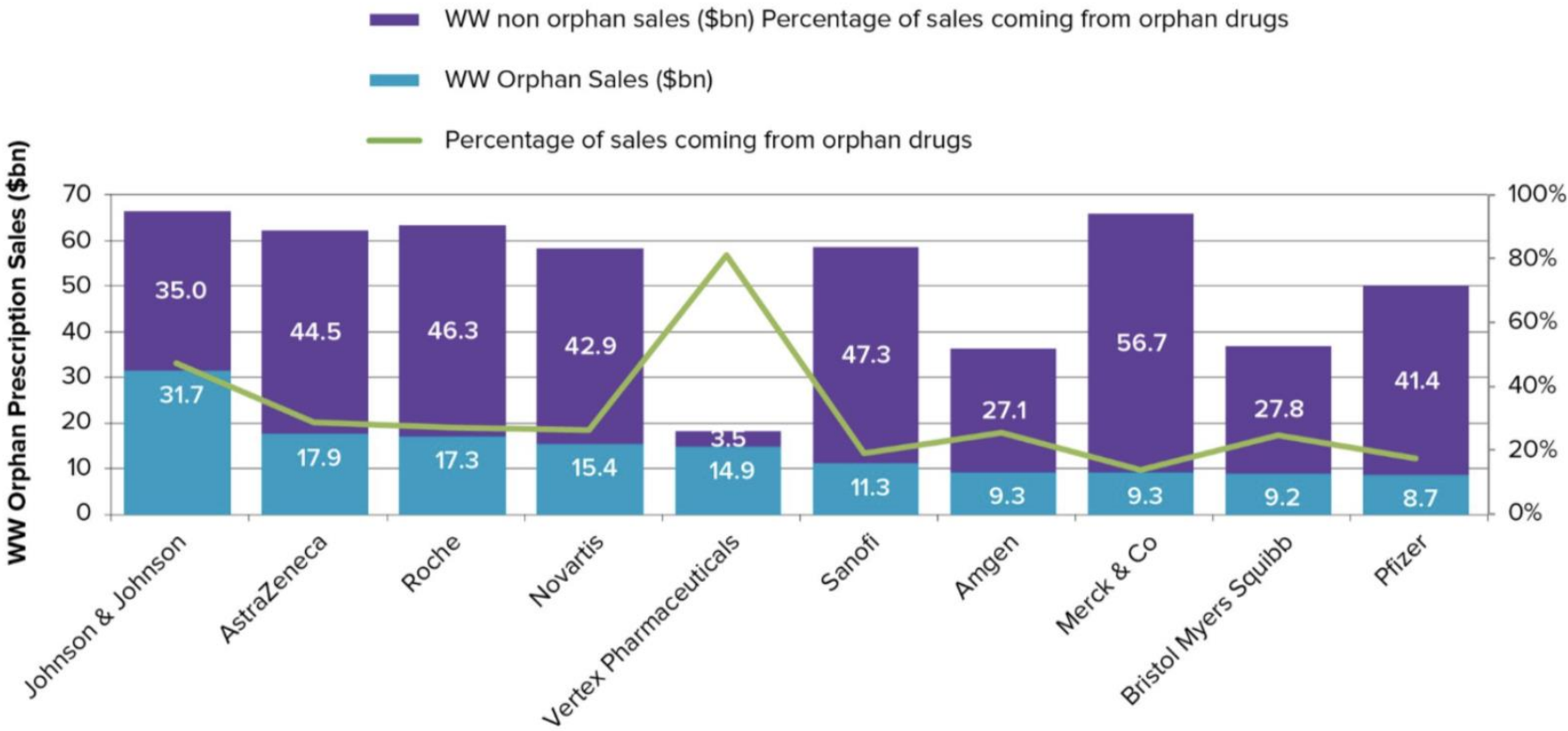
3.FDA – Orphan designation: Overview, 2024.

4.Jones Day. Congress Expands Orphan Drug Exemptions from Medicare Price Negotiations. July 2025.

Big Orphan Pharma



Top 10 companies in Orphan Prescription Drug Sales by 2030



Global Pharma Revenue¹
>50%


Sales²
20%

Premium Pricing³
17x

1. FDA – Orphan Pharma Report, 2025
2.EMA – Orphan designation: Overview, 2024
3.IQVIA Institute. Global Trends in Orphan Drug Pricing. 2022.

“Exclusivity is more than IP—it’s the window that gives small companies the breathing space to bring something truly meaningful to market.” - Partner, HealthCap

~\$86-123M

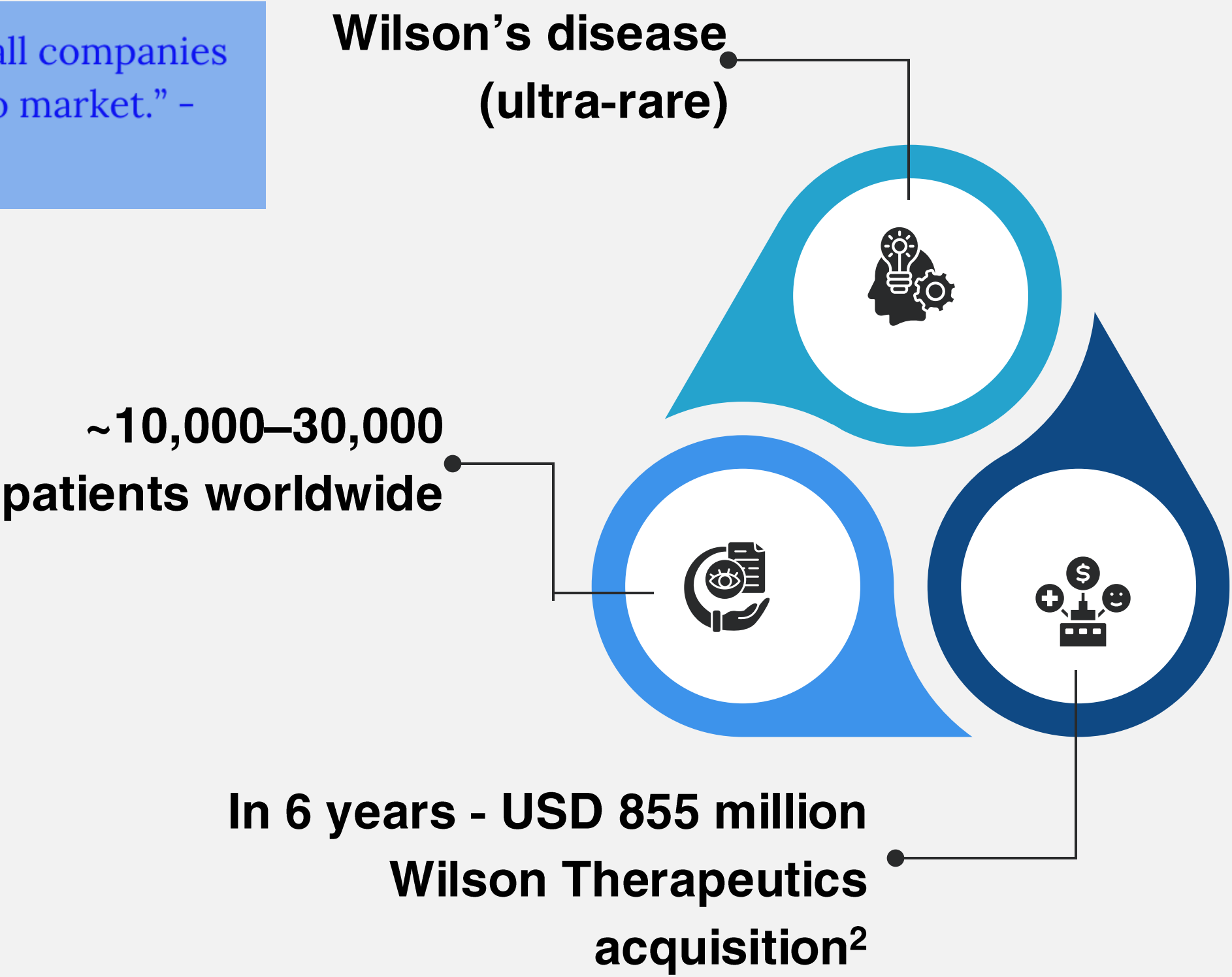


HealthCap’s total orphan investments (estimates)¹

8 Companies







1. HealthCap. Portfolio. 2024.
2.Alexion. Alexion to Acquire Wilson Therapeutics AB. Press Release, April 2018.

The Future: Precision Medicine & Orphan Drugs



Targeted Treatments

Genomic, environmental & lifestyle data



Enhanced Diagnostics

Gender, age, disease progression, genetic makeup

3 Key Insights



Orphan drugs are the fastest-growing segment in Pharma.



Big Pharma dominance & premium pricing drive strong investment value.



They are shaping the future of precision medicine.

Thank you!



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