WHAT 533 RARE-DISEASE PATIENT GROUPS SAY ABOUT PHARMA IN 2023-24

PRESS RELEASE EMBARGOED until
TUESDAY, AUGUST 20th 2024, 6am GMT

Contact: Alex Wyke
Tel: +44-(0)-7960-855-019
Email: report@patient-view.com

London, 20th AUGUST 2024

Overview

PatientView is pleased to publish 'The Corporate Reputation of Pharma—from the Perspective of Rare-Disease Patient Groups, 2023' (the 5th rare-disease edition in the annual 'Corporate Reputation' series). The 2023 review contains the results of a November 2023 to late-February 2024 survey, answered by 533 rare-disease patient groups.

2023's 533 respondent rare-disease patient groups were involved in multiple therapy areas —the largest representation being 79 respondent bleeding-disorders patient groups (PatientView is also publishing in August 2024 a separate report dedicated to 2023's bleeding-disorders results). The 533 stated that, collectively, and around the world, they had actively supported and served 2.8 million patients with a rare disease during 2023/24. [See end of press release for a profile of the respondent rare-disease patient groups.]

Continue reading, for details about …

- The headline industry-wide rare-disease results of the 2023 survey.
- The performance at corporate reputation of the 31 pharma companies included in the 2023 rare-disease analysis.

SUMMARY OF RESULTS

INDUSTRY WIDE:

Although fewer than 1 in 2,000 people have a rare disease, the number of people living with a rare disease totals around 300 million globally, according to the Lancet Global Health Report, 'The Landscape for Rare Diseases in 2024'. A figure as large as that represents a significant challenge for national healthcare systems worldwide.[1]

[1] https://www.thelancet.com/journals/langlo/article/PIIS2214-109X(24)00056-1/fulltext#:~:text=By%20definition%2C%20rare%20diseases%20affect,people%20live%20with%20rare%20diseases.

Drug innovation

Drug innovation in the field of rare diseases has expanded over the last four decades—not only due to advancements in the scientific understanding of many rare conditions, but also in reaction to the financial incentives that governments have provided to mitigate the prohibitively-expensive task of researching treatments for tiny populations of patients with a rare disease. Nevertheless, almost 95% of rare diseases still have no approved treatments.[2]

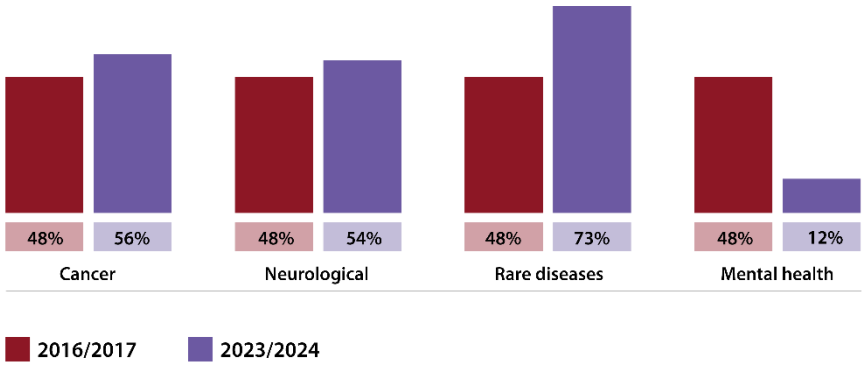
Even when rare-disease treatments are available, patients with a rare disease may be unable to access a timely diagnosis—the process of which can take anything up to four or five years, according to 2022 research from the European Commission.[3] The same EC study also assesses that half of Europe's 30 million people living with a rare disease are yet to receive a diagnosis.

The role of patient groups …

Patient groups have come to play a vital role in the lives of people living with a rare disease. PatientView estimates that as many as 21% of patient organisations worldwide (be they small national patient groups, or global alliances of patient groups) focus, in some way, on rare disease.[4] One of the strengths of any type of patient group is its ability to network; rare-disease patient groups have a reputation for being especially skilled at networking, and routinely use the technique to battle the challenges facing the patients they represent.

Unsurprisingly, pharma realises the importance of engaging with rare-disease NGOs. PatientView has found that the number of pharma companies (out of a sample of 50) partnering with ten or more rare-disease patient groups has risen by 25% between 2016 and 2024.[5]

Percentage of pharma companies with 10 or more patient-group partners—four specific broad categories of therapy area, 2016-2023
Source: PatientView [5]



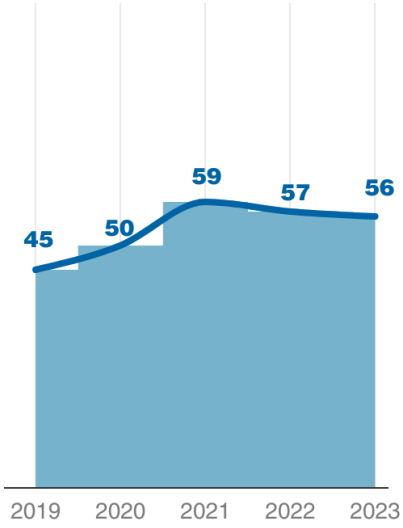
… and their views on pharma

Some 56% of rare-disease patient groups responding to 2023's 'Corporate Reputation of Pharma' survey rated the pharma industry's reputation as either "Excellent" or "Good", compared to 57% saying the same in 2022. However, 2023's respondent rare-disease patient groups also saw biotechnology companies as holding the highest reputation out of

[2] . https://www.iqvia.com/blogs/2023/09/how-rare-diseases-add-up-making-them-a-high-public-health-priority
[3] https://projects.research-and-innovation.ec.europa.eu/en/horizon-magazine/long-journey-rare-disease-diagnosis
[4] 'The Corporate Reputation of Pharma, 2023, Global Edition', PattientView, April 2024

nine healthcare sectors in 2023 (just ahead of pharma), with 61% of rare-disease patient groups judging biotech's corporate reputation "Excellent" or "Good." This result reflects the progress that biotech has made in early-stage R&D in the field of rare diseases (with pharma focusing on later-stage development).[5]

The corporate reputation of the pharma industry, 2019-2023
Percentage of respondent rare-disease patient groups stating "Excellent" or "Good"



How good or bad the pharma industry was at carrying out specific activities, 2023 v. 2022
% of respondent rare-disease patient groups stating "Excellent" or "Good"

	2023	Versus 2022
Patient centricity	48	+2
Information	49	+2
Ensuring patient safety	56	±0
Innovation	63	+5
Products that benefit patients	62	±0
Transparency: pricing	21	+3
Transparency: clinical data	29	+1
Transparency: funding	29	−1
Integrity	47	+3
Patient-group relations	55	±0
Services 'beyond the pill'	40	+1
Fair pricing policies	14	+3
Engaging patients in R&D	29	+1
Access to medicines	33	+2

Advice to pharma from **2023's respondent** rare-disease patient groups

Despite recent efforts at becoming more patient centric among pharmaceutical companies that focus on rare diseases, a striking message emerged from the quotes offered to the 'Corporate Reputation' survey by 2023's respondent rare-disease patient groups—industry could be working still more closely with rare-disease patient groups.

One noticeable theme was that pharma ought to try harder to gain a deeper understanding of the patient groups themselves (and the patients associated with these organisations). The industry, say 2023's respondent rare-disease patient groups, also needs to become more transparent—both in its pricing, and in its funding of healthcare stakeholders. While legislation mandates disclosure of funding by the industry, the information published may not, in the opinion of rare-disease patient groups, be sufficiently visible.

Nonetheless, 2023's respondent rare-disease patient groups obviously value, and welcome, involvement with pharma, and seem to entertain a real desire for closer cooperation.

[5] https://www.ncbi.nlm.nih.gov/pmc/articles/PMC11109548/

"Patiënten veel meer betrekken bij de ontwikkeling van geneesmiddelen en serviceproducten."

["Involve patients much more in the development of medicinal products and service products."]
—National rare-disease patient group, Netherlands

« En se préoccupant par exemple de l'accès aux médicaments en pharmacie de retrocession : c'est difficile pour les malades de s'y rendre. En se préoccupant d'une manière générale à la vie en dehors de l'hôpital, faciliter la vie sociale etc. »

["Taking an interest in the issue of access to medicines from hospital pharmacies, for example. Patients find difficulty getting there. Taking an interest generally in life outside of hospital, facilitating patient's social lives, etc."]
—National rare-disease patient group, France

„Patienten bei der Definition ihres Unmet Medical Need einbeziehen; Patienten in das Design klinischer Studien miteinbeziehen (meaningful outcomes - meaningful to them); Preispolitik bzw. Preisgestaltung transparent machen. Vertrauen und Kalkulierbarkeit (für Kostenträger und Leistbarkeit für Gesundheitssysteme); Patienten nicht nur als PR- oder PA-*Plattform sehen und nutzen, sondern echte Patientenzentriertheit leben."*

["Involve patients in defining their unmet medical need. Involve patients in the design of clinical studies (meaningful outcomes—that is, meaningful to them). Make pricing policy, or pricing, transparent. Trust and calculability (for payers, and affordability for health systems). Don't just see, and use, patients as a PR or PA platform, but be truly patient centric."]
—National rare-disease patient group, Austria

Companies included in the rare-disease edition of the 2023 **'Corporate Reputation'** survey results

The 31 companies featured in the rare-disease results of the 2023 'Corporate Reputation of Pharma' survey were selected on the criteria of size of revenue, or, by request from companies or patient groups. They include:

• AbbVie • Amgen (including Horizon Therapeutics) • Astellas Pharma • AstraZeneca • Bayer • Biogen • BioMarin • Boehringer Ingelheim • Bristol Myers Squibb • Chiesi Farmaceutici (including Amyrt Pharma) • CSL Behring • Eli Lilly • Gilead Sciences • Grifols • GSK • Ipsen • Janssen (known as Johnson & Johnson Innovative Medicine after late 2023) • Lundbeck • Merck & Co / MSD • Merck KGaA / EMD Serono • Novartis • Novo Nordisk • Octapharma • Pfizer • PTC Therapeutics • Roche / Genentech / Chugai • Sanofi • Sarepta Therapeutics • Servier • Takeda • UCB • Vertex.

Issues of importance to patient groups—as defined by patient groups

INDIVIDUAL COMPANY FINDINGS, 2023—AND THE FASTEST RISERS IN THE RANKINGS, 2023 v. 2022

The top-three rankings for corporate reputation in the field of rare disease in 2023 (out of all 31 companies)—as assessed by respondent rare-disease patient groups familiar with the company:

- **1st**, Roche
- **2nd**, BioMarin
- **3rd**, Amgen

The top-three rankings for corporate reputation in the field of rare disease in 2023 (out of 28 companies)—as assessed by respondent rare-disease patient groups **working** with the company:

- **1st**, Roche
- **2nd**, Amgen
- **3rd**, BioMarin

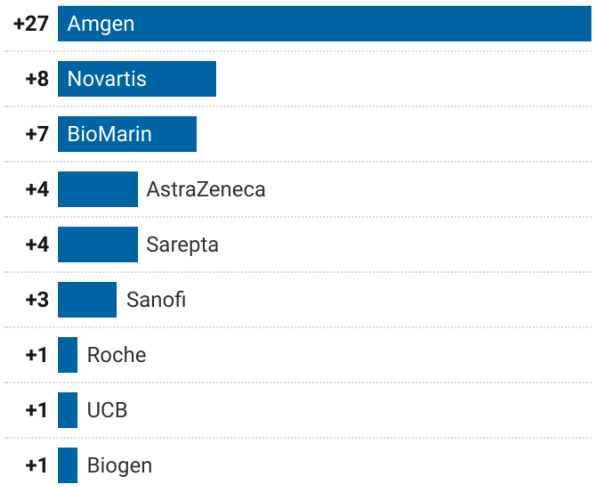
The top-three rankings for corporate reputation in the field of rare disease in 2023 (out of 15 'big-pharma' companies)—as assessed by respondent rare-disease patient groups **familiar** with the company:

- **1st**, Roche
- **2nd**, Amgen
- **3rd**, Sanofi

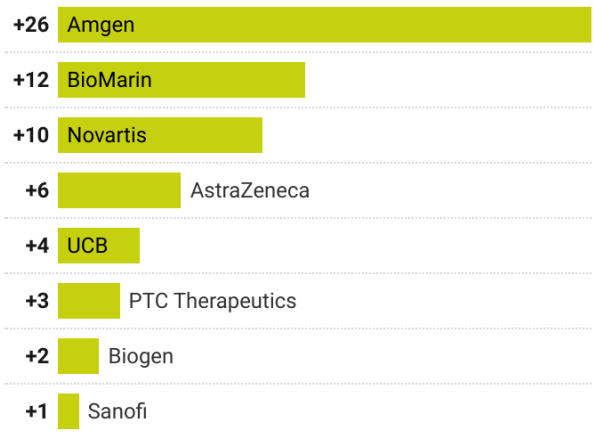
The top-three rankings for corporate reputation in the field of rare disease in 2023 (out of 13 'big-pharma' companies)—as assessed by respondent rare-disease patient groups **working** with the company:

- **1st**, Roche
- **2nd**, Amgen
- **3rd**, AstraZeneca

The companies rising the most in the upper rankings (out of all 31 companies), 2023 v. 2022
—as assessed by respondent rare-disease patient groups familiar with the company



The companies rising the most in the upper rankings (out of 28 companies), 2023 v. 2022 —as assessed by respondent rare-disease patient groups working with the company



Finally, PatientView would like to thank the 533 rare-disease patient groups that gave up their time to respond to the 2023 **'Corporate Reputation of Pharma' survey.**

The respondent rare-disease patient groups feel that the sharing of their evaluation and experiences on whether the pharma industry (and individual pharma companies) meets patient needs and expectations will help the industry gain valuable insights into improvement.

Because many of the 31 pharma companies featured in the 2023 'Corporate Reputation of Pharma' rare-disease edition are currently building strategies around patients, the respondent feedback provided by the survey results can influence company models and approaches, enabling closer alignment with patient needs and perspectives.

For further information on this report, please use contact details below.

END OF PRESS RELEASE

Contact: Alex Wyke
Tel: +44-(0)-7960-855-019
Email: report @ patient-view.com

Appendix

Profile of respondent rare-disease patient groups, 2023—by regional geographic headquarters (Africa, Asia-Pacific, Europe, North America, Latin America)
Percentage of respondent rare-disease patient groups

Profile of respondent rare-disease patient groups, 2023—by specialty
Number of respondent rare-disease patient groups