NOTIFICATION OF NEW PUBLICATION: What 154 rare-disease patient groups think of 28 pharma companies in 2019

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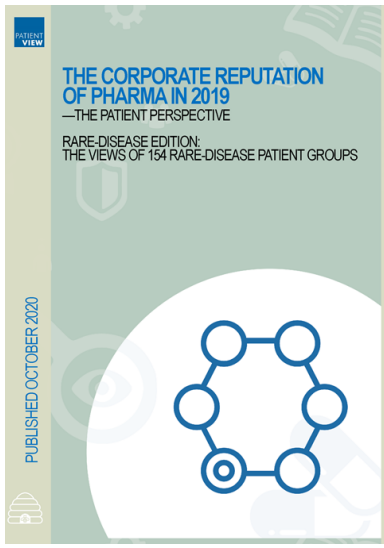


This is the 1st edition of 'The Corporate Reputation of Pharma - from the Perspective of Rare-disease Patient Groups'. These 2019 results are drawn from a survey of rare-disease patient groups, conducted November 2019 - February 2020.

Note: *The PatientView 2019 'Corporate-Reputation' survey took place largely before the Covid crisis became global.*

Specialties of respondent rare-disease patient groups, 2019

■ Achondroplasia (dwarfism) ■ Adult soft-tissue sarcoma ■ Amyloidosis ■ Angelman syndrome ■ Behcet's disease ■ Congenital deaf-blindness ■ Congenital disorders of glycosylation ■ Congenital/genetic red blood-cell diseases ■ Diseases of the ocular fundus ■ Fabry disease ■ Galactosemia ■ Gastrointestinal stromal tumour (GIST) ■ Gaucher disease ■ Guillain-Barre syndrome ■ Haemochromatosis ■ Hepatolenticular degeneration (HLD) ■ Hereditary angioedema ■ Hereditary endocrine tumours ■

About the 2019 survey of rare-disease patient groups

- **Country headquarters**. The rare-disease patient groups came from 44 countries.
- **Therapy areas**. The 154 covered a spectrum of rare diseases, including rare cancers [see accompanying list].
- **Geographic remit**. The respondent rare-disease patient groups had the following geographic remits: 72% had a national geographic remit; 12% regional (an area within one country);

Huntington's disease ■ Jacobsen syndrome ■ Lysosomal depot diseases ■ Marfan syndrome ■ Meretoja syndrome ■ Mucopolysaccharidosis ■ Myasthenia gravis ■ Neuroendocrine tumours ■ Neurofibromatosis ■ Peroxisomal disease ■ Phenylketonuria (PKU) ■ Pleural and peritoneal mesothelioma ■ Porphyrias ■ Prader-Willi syndrome ■ Rare bone diseases ■ Rare metabolic disorders ■ Rare chronic skeletal diseases ■ Rett syndrome ■ Sarcoidosis ■ Trisomy 21 ■ Tuberous sclerosis complex (TSC) ■ Tumours of the thymus gland ■ Waldenstrom macroglobulinemia ■ Wilson's disease (WD) ■ Wolfram syndrome

12% an international remit; and 3% local.

On the relationships that rare-disease patient groups had with pharma, 2019

- 61% of the 154 rare-disease patient groups responding to the 2019 survey worked with at least one pharma company. However, most companies worked with only a few rare-disease patient groups. The exceptions were Novartis, Pfizer, Roche/Genentech, Sanofi, and Takeda/Shire—all of which worked with 20 or more rare-disease patient groups [see table, below].

The 28 companies included for assessment in the 2019 rare-disease 'Corporate-Reputation' analyses (in alphabetical order):

AbbVie | Allergan | Amgen | Astellas | AstraZeneca | Bayer | Biogen | Boehringer Ingelheim | Bristol Myers Squibb | Celgene* | Chiesi Farmaceutici | CSL Behring | Eli Lilly | Gilead | GSK | Ipsen | Janssen | Merck & Co/MSD | Merck KGaA/EMD Serono | Novartis | Novo Nordisk | Pfizer | Roche/Genentech | Sandoz | Sanofi | Takeda/Shire | Teva | Vertex

* Celgene has been included as a separate entity in the 2019 results because the company's acquisition by Bristol Myers Squibb was only completed in November 2019, and the 2019 'Corporate-Reputation' survey is intended to reflect the views of patient groups throughout 2019.

Respondent rare-disease patient groups—their familiarity, and partnerships, with 28 companies, 2019

Company	Number familiar with	Number worked with
AbbVie	36	8
Allergan	20	1
Amgen	40	9
Astellas	22	1
AstraZeneca	49	8
Bayer	80	13
Biogen	39	5
Boehringer Ingelheim	35	5
Bristol Myers Squibb	38	5
Celgene	38	8
Chiesi Farmaceutici	24	5
CSL Behring	24	7
Eli Lilly	46	4
Gilead	24	3
GSK	65	12
Ipsen	30	9
Janssen	47	8
Merck & Co/MSD	35	6
Merck KGaA/EMD Serono	36	5
Novartis	104	38
Novo Nordisk	35	7
Pfizer	95	35
Roche/Genentech	82	21
Sandoz	38	3
Sanofi	79	30
Takeda/Shire	66	28
Teva	32	5
Vertex	22	3

What this report contains

Industry-wide analyses: The 2019 rare-disease 'Corporate-Reputation' report examines issues of importance to rare-disease patient groups, including three subjects that dominated the patient-group/pharma landscape in rare-diseases in 2019 ...

• pharma relationships with rare-disease patient groups; • pharma's investment in the R&D of treatments for rare diseases; and • patient engagement in R&D.

Analyses are reinforced by extensive feedback from 2019's respondent rare-disease patient groups [found in Appendix I of the report], organised according to the countries of the respondent patient groups.

Individual company analyses: The 28 pharma companies are reviewed by 2019's 154 respondent rare-disease patient groups for overall corporate reputation, and for performance at 12 individual indicators of corporate reputation.

The 12 indicators used to measure corporate reputation from a patient perspective

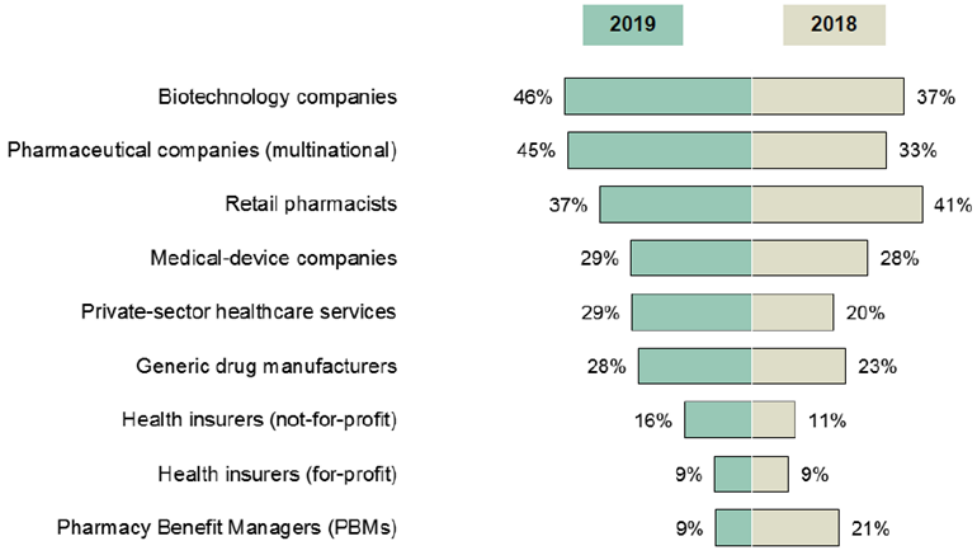
1. Patient centricity.
2. Patient information.
3. Patient safety.
4. High-quality products.
5i. Transparency: pricing.
5ii. Transparency: clinical-trial data.
5iii. Transparency: funding of stakeholders.
6. Integrity.
7. Quality of relationships with patient groups.
8. Providing services 'beyond the pill'.
9i. Engaging patients in research.
9ii. Engaging patients in development.

Key industry-wide findings for rare diseases, 2019

The corporate reputation of the pharma industry as a whole among patient groups specialising in rare diseases

2019's respondent rare-disease patient groups were more positive about the pharmaceutical industry's corporate reputation than in 2018. 45% of 2019's respondent rare-disease patient groups described the industry's corporate reputation as "Excellent" or "Good", compared with 33% saying the same in 2018.

The corporate reputation of the pharmaceutical industry, rare diseases, 2019 v. 2018—compared with eight other healthcare sectors (Percentage of respondent rare-disease patient groups stating "Excellent" or "Good")



However, respondent rare-disease patient groups also demonstrated signs of concern about the performance of the pharma industry as a whole at key activities in 2019. Aside from the issue of patient safety, less than half of the respondent rare-disease patient groups thought the pharmaceutical industry "Excellent" or "Good" at a range of activities of importance to patients, including: **innovation** (45%); and **the provision of high-quality products** (44%). 2018's equivalent figure for providing high-quality products was higher, at 51%. Less than 10% of the rare-disease patient groups thought the industry "Excellent" or "Good" in 2019 at **fair pricing policies.**

The comments provided to the 2019 survey by respondent rare-disease patient groups highlighted several reasons why the rare-disease patient-advocacy community is less happy with the pharmaceutical industry than patient groups of some other therapy areas.

On relationships between patient groups and pharma. The feeling among respondent rare-disease patient groups was that pharmaceutical companies have relatively few working partnerships with rare-disease patient groups (a fact borne out by the data collected in this survey [see table, above]), and that this is because rare diseases are not commercially interesting enough to justify the development of treatments. As Leukodystrophy Australia noted: *"It is our perception that pharma companies do not reach out to us, because we do not have the numbers to support financial gain."*

On research and development. Several respondent rare-disease patient groups requested the pharma industry to do more to embrace rare diseases. According to the Federação Brasileira de Hemofilia [Brazilian Federation of Haemophilia] (FBH): "*As empresas farmacêuticas devem apresentar-se ao nosso país e órgãos governamentais com propostas de estudos clínicos que auxiliam no desenvolvimento de medicamentos para proporcionar melhor qualidade de vida e segurança às pessoas com Hemofilia A e B, von Willebrand e outras coagulopatias hereditárias.*"("Pharmaceutical companies need to present our country and government bodies with proposals for clinical studies that aid in the development of drugs to provide better quality of life, and safety for patients with haemophilia A and B, von Willebrand disease, and other and other hereditary coagulopathies.")

On patient involvement in research and development. Comments from respondent rare-disease patient groups show that these organisations are strongly motivated to be involved throughout the research-and-development process—from confirming unmet needs, trial design and execution, through into the product lifecycle. Thus, Genetic Alliance Australia said: "*Have consumers or patients as part of trials development. More patient-reported outcomes, and experiences recorded as part of trials, and for ongoing treatment when the trial is completed.*"

Key company findings for rare diseases, 2019

Sanofi was ranked overall 1st out of 28 companies for corporate reputation in 2019 by the 79 respondent rare-disease patient groups familiar with the company. Sanofi was also ranked overall 1st out of 5 companies for corporate reputation in 2019 by its 30 respondent partner rare-disease patient groups.

Pfizer was ranked overall 2nd out of 28 companies for corporate reputation in 2019 by the 95 respondent rare-disease patient groups familiar with the company. Pfizer was also ranked overall 2nd out of 5 companies for corporate reputation in 2019 by its 35 respondent rare-disease patient-group partners.

Takeda/Shire was ranked overall 3rd out of 28 companies for corporate reputation in 2019 by the 66 respondent rare-disease patient groups

familiar with the company. Takeda/Shire was ranked overall 5th out of 5 companies for corporate reputation in 2019 by its 28 respondent rare-disease patient-group partners.

Comparing just the 14 largest pharma companies ('big pharma'), rare diseases, 2019 v. 2018

To enable peer-to-peer comparisons of the results, PatientView also recalculates overall rankings for the 12 indicators of corporate reputation for just the 14 largest, multinational, multi-therapy pharma companies. These 'big-pharma' results provide a different perspective on how the largest pharmaceutical companies fare for corporate reputation—enabling true peer-to-peer analyses.

For ***further information*** on PatientView's latest publication, 'The Corporate Reputation of Pharma—from the Perspective of Rare-disease Patient Groups, 2019', please use the contact details at the top.

To download the publication's contents, list of tables and charts, and sample materials, please click below:

LINK TO SAMPLE PAGES OF 2019's RARE-DISEASES ANALYSIS

~END OF NOTIFICATION~

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