

Important Vocab for the Editorial

1. **vital** (adjective) – key, essential, important.
2. **binding** (adjective) – compulsory, obligatory, imperative, mandatory, necessary.
3. **welfare state** (noun) – a form of government in which the state protects and promotes the economic and social well-being of the citizens, based upon the principles of equal opportunity, equitable distribution of wealth, and public responsibility for citizens unable to avail themselves of the minimal provisions for a good life. A system wherein the government agrees to underwrite certain levels of employment, income, education, medical, social security and housing for all its citizens.
4. **secure** (verb) – obtain, acquire, gain, get.
5. **wellbeing** (noun) – a healthy state/condition.
6. **irrespective of** (adjective) – notwithstanding, without regard for, regardless of.
7. **constitute** (verb) – comprise, represent, amount to, add up to.
8. **critical mass** (noun) – a minimum number of people in relation to something.
9. **National Policy for Rare Diseases 2021 (NPRD)** (noun) – The National Policy for Rare Diseases 2021 (NPRD) was notified on March 31. It aims to lower the high cost of treatment for rare diseases, with an increased focus on indigenous research and local production of medicines. A provision for financial support up to ₹20 lakh under the Umbrella Scheme of Rastriya Arogya Nidhi is proposed for the treatment of those rare diseases that require a one-time treatment (diseases listed under Group 1 in the rare disease policy).
10. **intervention** (noun) – the process of intervening in something; involvement, interference.
11. **peg** (verb) – introduce, draw attention to (ideas/opinions).
12. **up to** (phrase) – indicating a maximum amount.
13. **crowdfunding** (noun) – relating to a method of getting a large number of people to give small amounts of money to finance a business project or cause.
14. **mechanism** (noun) – structure, system, procedure/method.
15. **registry** (noun) – record book, register, log, official list.
16. **lobby** (noun) – a group of people attempting to influence (the government / its official group/elected legislators) to create the actions, policies, or decisions to help a particular organization.
17. **sorely** (adverb) – severely, desperately, badly, critically.
18. **broadly** (adverb) – generally, usually, mainly.
19. **marker** (noun) – indicator, pointer, index.
20. **prevalence** (noun) – it refers to the number of cases of a disease that are present in a particular population at a given time whereas “incidence” refers to the number of new cases that develop in a given period of time.
21. **Organization for Rare Diseases India (ORDI)** (noun) – a national umbrella organization representing the collective voice of all patients with rare diseases in India, set up as a section 25 non-profit company in India.
22. **inherited** (verb) – hereditary, inherent, congenital; derived/obtained genetically.

23. **autoimmune** (disease) (adjective/noun) – it is used to describe conditions in which the body's immune system – which is supposed to be defending the body against viruses, bacteria and other invaders – is thought to be involved in causing or perpetuating illness.
24. **disorder** (noun) – disease, infection, illness.
25. **malformation** (noun) – (of a disease or physical) abnormality, deformity.
26. **Hirschsprung's disease** (noun) – a condition that affects the large intestine (colon) and causes problems with passing stool. The condition is present at birth (congenital) as a result of missing nerve cells in the muscles of the baby's colon.
27. **Gaucher disease** (noun) – a genetic disorder where fat-laden Gaucher cells build up in areas like the spleen, liver and bone marrow.
28. **Cystic fibrosis** (CF) (noun) – an inherited disorder that causes severe damage to the lungs, digestive system and other organs in the body.
29. **Muscular dystrophy** (noun) – a group of diseases that make muscles weaker and less flexible over time. It is caused by a problem in the genes that control how the body keeps muscles healthy.
30. **Lysosomal Storage Disorders (LSDs)** (noun) – Lysosomal storage diseases are inherited metabolic diseases that are characterized by an abnormal build-up of various toxic materials in the body's cells as a result of enzyme deficiencies. There are nearly 50 of these disorders altogether, and they may affect different parts of the body, including the skeleton, brain, skin, heart, and central nervous system.
31. **evolve** (verb) – alter, change, transform gradually; develop, progress, advance gradually.
32. **in the hope that** (phrase) – with the hope that something will take place.
33. **commercialisation** (noun) – the process of doing something to get financial benefit.
34. **drum up** (phrasal verb) – try to get support (by urging/convincing/pressuring someone).
35. **long-fought** (adjective) – demanding, challenging, arduous, taxing.
36. **short of** (phrase) – less than.
37. **mandate** (noun) – ruling, ordinance, law, instruction, directive, order; approval, ratification, endorsement.
38. **prohibitive** (adjective) – excessively high, exorbitant, excessive.
39. **categorise** (verb) – class, group, list.
40. **Drug Controller General of India (DCGI)** (noun) – Drugs Controller General of India (DCGI) is a department of the Central Drugs Standard Control Organization (CDSCO) of the Government of India responsible for approval of licences of specified categories of drugs such as vaccines, and etc. in India. Drugs Controller General of India, comes under the Ministry of Health & Family Welfare. DCGI also sets standards for the manufacturing, sales, import, and distribution of drugs in India.
41. **point out** (phrasal verb) – identify, mention, indicate, draw attention to.
42. **work out** (phrasal verb) – develop, form, initiate/establish.
43. **halve** (verb) – reduce by half (50%).
44. **set aside** (phrasal verb) – save, reserve, put aside.
45. **substantial** (adjective) – considerable, significant, large.
46. **corpus** (noun) – (in finance) total amount of money; collection.

47. **roll out** (phrasal verb) – launch, introduce, organize, inaugurate (something officially).
48. **cement** (verb) – establish firmly.

A good start: On rare diseases and government support for treatment

Fund support from government is vital for continual treatment of those with rare diseases

It is binding on a welfare state to take care of every single citizen. Securing the wellbeing of every one, particularly those unable to help themselves, irrespective of whether they constitute a critical mass or not, is important. The recent notification of the [National Policy for Rare Diseases 2021](#) after various interventions, including the court, is pegged on this principle of inclusion. A good start, it offers financial support for one-time treatment of up to ₹20 lakh, introduces a crowdfunding mechanism, creates a registry of rare diseases, and provides for early detection. In its final form, however, the policy has left the rare diseases lobby sorely disappointed on a crucial note. Rare diseases are broadly defined as diseases that infrequently occur in a population, and three markers are used — the total number of people with the disease, its prevalence, and the availability/non-availability of treatment options. WHO defines rare disease as having a frequency of less than 6.5-10 per 10,000 people. As per an estimate, there are 7,000 known rare diseases with an estimated 300 million patients in the world; 70 million are in India. According to the Organization for Rare Diseases India, these include inherited cancers, autoimmune disorders, congenital malformations, Hirschsprung's disease, Gaucher disease, cystic fibrosis, muscular dystrophies and Lysosomal Storage Disorders (LSDs).

Much of the effort in the sector, from the medical side, has been to evolve formal definitions, in the hope that it would support the development of and commercialisation of drugs for treatment, and improve funding for research on rare diseases. Patient support groups have worked towards drumming up funding assistance for the treatment — one time or continual. The notification of the Policy comes as a logical conclusion to a long-fought battle, and yet, stops short of delivering the complete mandate. As per the Policy, diseases such as LSD for which definitive treatment is available, but costs are prohibitive, have been categorised as Group 3. However, no funding has been allocated for the immediate and lifelong treatment needs, for therapies already approved by the Drugs Controller General of India. Experts point out that the costs to help already-diagnosed patients might be in the range of ₹80-₹100 crore annually. If the Centre can

extend the cost-sharing agreements that it has worked out with Kerala, Tamil Nadu and Karnataka, with other States too, its share of the annual costs will be halved. The Centre can, however, still set aside a substantial corpus to fund life-saving treatments, even as it rolls out the policy. Doing so will not only complete a job well begun — even if not yet half done — but also cement its commitment towards the welfare of every single citizen in India.

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