Geneva, 3 January 2025

To:

Member States' Missions to the World Health Organization

Urgent Need to Strengthen the WHO Resolution on Rare Diseases to Ensure Equitable Access to Treatment

Esteemed Ambassadors and UN Missions,

We, the undersigned civil society organizations, are writing to express our deep concerns regarding the current draft resolution on rare diseases to be discussed during the WHO's 156th Executive Board meeting. While we appreciate the initiative to address rare diseases as a global health priority, we believe the resolution, in its present form, does not adequately address the fundamental barriers to effectively face up to rare diseases i.e. equitable access to life-saving treatments, particularly in developing countries.

The challenges posed by rare diseases are considerable. At present, 95% of rare diseases lack approved treatments, and where treatments exist, they are exorbitantly priced rendering them inaccessible to most people living with rare diseases (PLWRD). The monopolistic pricing strategies adopted by pharmaceutical companies drive the cost to unreasonably high levels. For instance, Trikafta, the drug for treating cystic fibrosis, is priced at $327,000 per year, despite independent research estimating its production costs to be approximately $5,676. Similarly, Risdiplam, the drug used for treating spinal muscular atrophy (SMA) is priced at $80,000 annually, though its production costs can be as low as $34 per year.

Moreover, pharmaceutical companies frequently exploit regulatory frameworks to prolong market exclusivity and restrict competition. Strategies such as "salami slicing," wherein diseases are divided into narrower sub-categories to obtain multiple orphan drug designations, and "indication stacking," where additional uses for existing drugs are patented to extend exclusivity, contribute to the evergreening of patents and maintain artificially high prices. The case of CAR-T cancer therapies is illustrative: these treatments cost between $260,000 and $350,000 per patient in commercial markets. In Spain and India publicly developed CAR-T therapies are available at a significantly lower cost.

Abuse of patent monopolies is also one of the major contributing factors to the high prices of these medicines, preventing low-cost generics from entering the market. Due to the abusive patent practices, many developing countries with robust generic manufacturing capacities face restrictions in manufacturing affordable versions of these medicines. It is not uncommon for pharmaceutical companies to obtain patents

in countries where they do not offer the drug, thereby denying patients access to essential treatments. In India, for instance, Vertex–the manufacturer of Trikafta–has obtained secondary patents, erecting barrier to generic production, but not registering the drug for marketing in India. This leaves an estimated 45,000 cystic fibrosis patients without access. In contrast, India's refusal to grant patents for CAR-T therapies has facilitated the development of domestically produced alternatives at substantially lower costs ($47,000 versus $500,000–$600,000 in the United States).

The high cost of patented medicines/treatments has resulted in the denial of access to efficacious medicines for PLWRD, especially those living in developing countries.

Given these significant issues, we strongly urge Member States to strengthen the resolution by incorporating the following elements:

- to explicitly mandate the Director-General of WHO to prepare a report comprehensively mapping the availability and affordability of medicines/treatment as well as diagnostics for rare diseases, as part of the preparatory work towards the development of a Global Action Plan for Rare Diseases;
- urging Member States to use TRIPS flexibilities to address high prices emanating from intellectual property monopolies and promote affordable access; a clear precedent for this can be found in resolution WHA67.6 on hepatitis.
- stronger commitments by WHO Members to ensure transparency in medicine pricing, taking into account resolution WHA72.8.

It is imperative that this resolution moves beyond general acknowledgements and establishes concrete measures to ensure equitable access to treatment for rare diseases. A weak resolution, devoid of substantive and meaningful commitments, will only perpetuate existing inequities and fail the millions of patients who are in need, in developing and developed countries.

We, therefore, strongly urge the WHO Executive Board Members as well as other Member States to introduce the above elements and to adopt a resolution that prioritizes affordable access to efficacious medicines and treatment for rare diseases over commercial interests.

Sincerely,

Global

1. Aequa, Think Space On Equity And Economic Justice For Health
2. Asem Catalunya Associació Catalana de Malalties Neuromusculars
3. Health Action International (HAI)
4. Health Global Access Project
5. International Baby Food Action Network
6. International Treatment Preparedness Coalition (ITPC)
7. Policies for Equitable Access to Health
8. Public Services International
9. Religious of the Sacred Heart of Mary NGO.
10. Society for International Development (SID)
11. Third World Network (TWN)

Regional

12. Association of Women of Southern Europe AFEM
13. Health Action International Asia Pacific

National

14. Access to Medicines Research Group, *China*
15. Acción Internacional para la Salud, *Peru*
16. Asociación por un Acceso Justo a los Medicamentos (AAJM), *Spain*
17. Association for Proper Internet Governance, *Switzerland*
18. Cancer Alliance, *South Africa*
19. Cornelia de Lange Syndrome Family, *Indonesia*
20. Civil Society Coalition on Transport in Uganda (CISCOT), *Uganda*
21. Crisis Home KL, *Malaysia*
22. Cure SMA, *Bangladesh*
23. Cure SMA Québec, *Canada*
24. CureSMA Foundation of India, *India*
25. Curesma South Africa, *South Africa*
26. Drug Action Forum - Karnataka, *India*
27. Dumaic Global Health, *Uganda*
28. Egyptian Initiative for Personal Rights (EIPR), *Egypt*
29. Evangelical Lutheran Church in America, *United States*
30. Foundation for Integrative AIDS Research (FIAR), *United States*
31. Fundación IFARMA, *Colombia*
32. Fundacja SMA, *Poland*
33. Fundame, *Spain*
34. Health and Nutrition Development Society, *Vietnam*
35. Indonesia for Global Justice, *Pakistan*
36. Indonesia for Global Justice (IGJ), *Indonesia*

37. Indonesia Partnership for Health, *Indonesia*
38. Indonesia Rare Disorders, *Indonesia*
39. Indonesian CdLS Family, *Indonesia*
40. Indonesian Spinal Muscular Atrophy Community, *Indonesia*
41. Institut de réadaptation en déficience physique de Québec, *Canada*
42. Jannatul Hurin Nisa, *Indonesia*
43. Just Treatment, *United Kingdom*
44. Khalil Khamis, *Malaysia*
45. Khulna Mukti Seba Sangstha, *Bangladesh*
46. Land for Hope, *Lebanon*
47. Lebanese Association for NMD (Land), *Lebanon*
48. Low Cost Standard Therapeutics, *India*
49. Madhira Institute, *Kenya*
50. Malaysian Rare Disorders Society / Mywatch, *Malaysia*
51. Malaysian Women's Action for Tobacco Control and Health (MyWATCH), *Malaysia*
52. Penabulu Foundation, *Indonesia*
53. People's Health Movement, *Nepal*
54. People's Health Movement, *Burundi*
55. Persatuan Kebajikan Ceriajaya Kuala Lumpur Dan Selangor (Wecarejourney), *Malaysia*
56. Pharmaceutical Accountability Foundation, *Netherlands*
57. Prayas Center for Health Equity, *India*
58. Public Eye, *Switzerland*
59. Rekat Peduli Indonesia, *Indonesia*
60. Salud por Derecho, *Spain*
61. Salud y Farmacos, *United States*
62. Sankalp Rehabilitation Trust, *India*
63. Siva SK, *Malaysia*
64. SMA Philippines, *Philippines*
65. Soweto Youth Organization, *Kenya*
66. TB Care Network, *Indonesia*
67. Vietnamese Organization for Rare Diseases, *Vietnam*
68. Working Group on the Pandemic Agreement and Amendments to the IHR, *Brazil*
69. Yayasan Hipertensi Paru, *Indonesia*