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Subject: Contribution submission - UNSG high-level panel on access to medicines

This is a personal submission from John Forman, Rare Disease Advocate, from New Zealand. I have 41 years' experience as a parent of twins with a rare disease that involves significant and progressive physical and intellectual disability, and 25 years of advocacy roles in health and disability services in New Zealand, plus substantial roles in international rare disease advocacy networks spanning more than a decade.

Submission summary

1. This submission focusses on a narrow but important, yet often marginalised section of all populations in need of affordable access to medicines – those who have a rare disease. They exist in numbers that are very substantial in all countries, about 6% of all populations (Eurordis 2005, page 3), whether in advanced or developing nations, yet their needs for healthcare generally, and specialised "orphan" medicines in particular, are often in a serious state of neglect relative to the average patient in their own country or community.

2. It is proposed that the particular needs of the rare disease population are an essential aspect of what the right to health and universal healthcare should include. Regardless of their needs and rights as individuals and groups of patients within their own communities, the disadvantage of rarity makes them a significantly neglected population, just like those whose health status is negatively affected by indigenous or other minority status, or those with significant socio-economic disadvantage, or remote locations, across all nations. This situation demands that special attention should be given to the needs of rare disease populations in healthcare in general, as well as in access to medicines specifically.

3. Furthermore, the needs of rare disease patients for access to orphan medicines, offers a sometimes extreme yet very informative example of the dynamics and outcomes of the tensions between intellectual property rights and the right to health which this consultation seeks to explore.

4. I suggest that the tension between intellectual property right and the right to health, as presented in the request for submissions as largely a dichotomous problem, is too simplistic an analysis of the dilemma under discussion, whether in respect of medicine access or healthcare generally. While this tension is real, analysis of actual experiences in a number of countries (NZ is a classic example) shows that there is a significant third factor involved in the mix. That factor is the willingness, or otherwise, of governments to accept their obligations that flow from their citizens' right to health, and the resulting significant conflict of interest they often have in also being (in most situations) the funder of healthcare and medicine costs.

5. Experience shows that many countries, as payers of healthcare costs, are quick to use their sovereign powers to negate the rights of their citizens to orphan medicines for rare diseases, usually citing high costs and poor comparison against other possible healthcare investments. It is clear that the legal framework set up in many countries puts the right to health as subservient to the political decisions of government, (e.g. NZ Bill of Rights Act 1990) rather than elevating

the human rights framework to a level that guides and instructs the actions of governments. By contrast, if the right to health is expressed in a written constitution, there may be a greater likelihood that medicines and services will be provided. This third factor of governments' dual role of policy maker and funder, and the resulting conflict of interest, accentuates the dilemmas of access to medicines for those with rare diseases.

6. Given the existence of negotiation and pragmatic agreements in a number of countries to fund orphan medicines for rare diseases, it is apparent that positive outcomes are achievable, that compromises are made both by supplier and payer, and that the right to health is clearly given more practical effect by some governments than others. It is also apparent that medicine developers do make a number of concessions against their intellectual property rights when governments engage seriously with them to give effect to their citizens' right to health.

7. The policy solution I propose is an agency based in the high level reaches of the UN, WHO, or within the international human rights framework, with this agency charged with an intervention agenda to actively engage with governments and industry over medicine access in each country, with special attention given to access to orphan medicines for rare diseases. In dealing with individual governments, the agency would address their practical application of the right to health for their citizens, to encourage them to engage constructively with industry with the intention of gaining access to needed medicines, and to deal honourably with the substantial conflict of interest they have. In dealing with industry, they should seek negotiations on mechanisms that would facilitate access at affordable prices, including the use of tools such as differential pricing schemes, managed access schemes, and similar tools, to improve access while managing costs. The agency should also act as a broker to include patient advocacy and not-for-profit foundations as partners in its work with governments and industry, to achieve acceptable and sustainable solutions.

8. There are parallels here with moves to promote universal healthcare, especially under the Millennium Development Goals and the emerging Sustainable Development Goals, where international agencies successfully encouraged and motivated many countries to make quantum leaps with the extension of core healthcare services to previously underserved or neglected populations. There are parallels also in relation to security and trade, with high level agencies charged with leading and facilitating better outcomes for all.

9. The proposal outlined in this submission is suggested as the most likely and practical tool for impacting on the current policy incoherence in access to medicines, improving public health and giving practical effect to the human right to health, within any individual country. Of course the agency could be the same one(s) charged with promoting universal healthcare and other Sustainable Development Goals, but the critical point would be the acceptance of the intervention agenda outlined here, to ensure progress is real and not subject to lip service. This proposal is not suggested as a panacea for all medicine access issues in the world. It is focused solely on medicines for rare diseases.

10. Note, importantly, that the presence of an industry with intellectual property rights and often high charges, can lead to political and partisan stances on the part of many interested parties, including governments, officials, and academics. What is vital for success in this endeavor would be the principle of constructive engagement that leaves partisan views aside, and executive

function to act as a broker and mediator with successful negotiations the prime outcome sought.

Additional discussion points

11. My submission is based on ICORD's Yukiwariso Declaration which tracks the connection between the Universal Declaration of Human Rights, various international Covenants and Conventions that most governments have signed up to and which reinforce their commitment to the human right to health, and the influence of moral philosophy which is a strong force in most legal systems and public policy, and guidance in this case about how the right to health should be applied in practice. That Declaration states what governments ought to do, and gives strong encouragement to them to deliver. This call for submissions on what should be done at a policy level, offers the natural extension of the Yukiwariso's discussion into practical implementation steps at a global level.

12. There have been incentives since the 1980s, firstly in the US and then the EU from 2000, for the development of orphan drugs. These have been successful in bringing many new medicines to market. While price is a sticking point for some payers, it is notable that in the US the government is quite removed from the role of payer for most of the population. Despite the prices, the US seems to provide better access to orphan medical products for patients with rare diseases, than the socialized systems in the EU and elsewhere, despite historically low insurance cover for the US population. This seems to reinforce the point about the conflict of interest where government is also the payer.

13. There is considerable risk of distraction and diversion in any suggestion of using Health Technology Assessment models, transparent pricing systems, or other approaches to measuring value, as a basis for price negotiation. HTA on its own is primarily useful for comparing cost and benefits of alternative treatments or interventions for the same condition. If it is used to compare value of available interventions or treatments between different populations, or different diseases, it is highly likely to bias the outcomes against those affected by rare diseases. The calculations will mostly not find treatments for rare diseases cost-effective compared to other health interventions, especially medicines for common diseases. This highlights the value of HTA in certain situations but its very limited value in others, and shows the HTA and similar debates in the case of orphan medicines for rare diseases, to be a diversion from the core problem, rather than a potential solution.

14. There is also a significant risk to good decision-making in health if HTA is used as the prime consideration. Note especially the decision framework used until recently by Pharmac in New Zealand which gave excess emphasis to costs of health outcomes and budget management, but excluded any specific consideration of human rights or moral factors such as fairness and equity. Without counterbalancing moral and ethical considerations, HTA alone as the prime factor in decisions is likely to frequently do harm and conflict with the right to health. See my 2014 discussion on ethics, equity and community values in medicine decision making in New Zealand. Note that the resulting policies adopted by Pharmac did adopt a broader range of factors for consideration and established an orphan drugs fund, indicating some effect of the advocacy made, but the overall legislative mandate of the organisation remains restrictive and is a continuing problem for access to medicines.

15. The work of the WHO in promoting use of HTA to assist with the attainment of universal healthcare has value in addressing the right to health only if there is specific inclusion of rights and moral factors in the decision making process. It is disappointing that the subsequent WHO report is silent on that point and that the work of that group is so dramatically light on representation form patient advocacy groups.

16. Compulsory licensing has been one mechanism widely discussed and sometimes implemented for the production of generic substitutes of essential medicines in some countries. Whatever the pros and cons of this as a tool to improve access, it should be noted that compulsory licensing is very unlikely to have much impact or feasibility in relation to orphan medicines, primarily because of the complexity of the biological systems required for manufacture of many orphan drugs.

17. Concern has been frequently expressed about the likely impact of orphan medicine products on overall health system sustainability. This may well be a red herring, as past concerns about catastrophic impact on healthcare systems of blood products and dialysis have been unfounded in the developed nations. In respect of costs of orphan medicines for rare diseases, the literature is divided over the likely future impacts and sustainability with Hutchings et al and Schey et al finding little concern about unmanageable impacts, while Kanters et al provide a contrary view. My own estimates suggest that the cost to New Zealand's health system to fund all of the orphan medicines available in the marketplace, but not funded here in 2014, would have been less than 3% of the entire community medicines budget. Further analysis shows that just a part of the medicine budget savings in New Zealand in the 2013-14 and 2014-15 financial years could have funded all the unfunded orphan medicines without any increase to the medicine budget, though the savings were in fact used for other purposes. At least as far as the OECD countries are concerned, there seems to be very weak evidence and mostly partisan views, that suggest the cost of orphan medicine products is not sustainable now or into the foreseeable future.

18. An important point to note in this discussion is the extent to which governments are constructing legal frameworks that place their elected representatives above the human rights framework. This cements their conflict of interest into the system to their own advantage. (See example in para 5 – the NZ Bill of Rights Act). Note that this approach has been praised in some quarters as a sound approach to human rights legislation (see Gledhill in the NZ Herald 1 October 2010), but there are very concerning aspects of this in regard to such a fundamental right as the right to health, when the government is so seriously conflicted in its dual roles. This should not be acceptable and the panel is encouraged to address this point with recommendations that the reverse situation should apply.

19. The dangers to the right to health that arises from Governments' conflicts of interest, is well demonstrated in the long running human rights case in New Zealand regarding support for family carers of disabled adult children. In the case recently reported in the NZ Herald the Crown is refusing to concede on the multiple victories that families have won in the Human Rights Tribunal and in the Courts. Note in particular the commentary by Law Professor Andrew Geddes which demonstrates the definite weakness of the human rights framework in such situations and the willingness of a government to manipulate things to its own advantage.

20. The essence of this proposal for an agency with an intervention agenda, is that it reflects some similar existing structures and agencies, and also reflects what has been done in the past for access to medicines in other situations, e.g. drugs for HIV, vaccine production and distribution. Though those particular projects involved charitable foundations which are unlikely to be much interested in orphan drugs access, they had significant political push for solutions behind them, and they worked in bringing multiple parties together to develop solutions. While perhaps not perfect solutions in those areas, those groups made significant progress in developing much better access for their medicines and diseases of interest, at an affordable price for governments. This proposal builds on a system that exists and has demonstrated some ability to work successfully.

My experience

21. I have 41 years' experience as a parent of twins with a rare disease that involves significant and progressive physical and intellectual disability, and 25 years of advocacy roles in health and disability services in New Zealand, plus substantial connections with international rare disease advocacy networks spanning more than a decade. Currently I am the president of ICORD (www.icord.se) the International Conference on Rare Diseases and Orphan Drugs. 17 years ago I established Lysosomal Diseases New Zealand (www.ldnz.org.nz) to provide information and support for affected families, and I am the Chair of that group. Just over 15 years ago I set up NZORD (www.nzord.org.nz) the NZ Organisation for Rare Disorders, which I led until my retirement last year. I have continuing roles in supporting a number of local and international rare disease advocacy groups, including being part of the establishment group for Rare Voices Australia (www.rarevoices.org.au) and still involved as RVA's acting Chair while board renewal is carried out.

22. My relevant academic experience includes a post-graduate course on Public Health Law & Public Health Ethics in 2008 from Otago University, lead author on ICORD's Yukiwariso Declaration on the need for worldwide policies and action plans for rare diseases, and various other articles, presentations, chapters and posters relating to rights and ethics in healthcare for rare diseases. The main ones can be found at https://www.researchgate.net/profile/John_Forman2/contributions

23. My policy experience in medicines access and rare diseases generally, revolves in particular around the challenges with the narrow legislative framework set up in New Zealand over the past 25 years to drive cost savings in medicines, and the negative impact this had regarding access to new medicines for most diseases groups, but for rare diseases in particular. I have led extensive analysis, reviews, submissions and appeals to our Ministry of Health, to Pharmac our drugs purchasing agency, and to political leaders, over my 15 years with NZORD. The results of this advocacy work is a new orphan drugs fund in New Zealand which is just starting to address the need for access to medicines for rare diseases, and a revised set of decision criteria adopted by our medicine funding agency, that broadens the range of factors to be taken into account. I have become very familiar with the legal, policy, ethical and social dimensions of medicine policy in NZ, and read extensively on similar issues in other countries. A variety of these experiences are recorded in the news section of the NZORD website http://www.nzord.org.nz/news/press_releases_and_submissions

24. I am making this as a personal submission as it is not realistic to claim to speak on behalf of the wide range of groups I am involved with on this matter. Even trying to seek endorsement from such a wide and diverse group of stakeholders would be challenging at the best of times, but impossible in the very tight timeframe for this submission. However, the points I make in this submission are based on the extensive engagement I have had with many hundreds of stakeholders in patient advocacy, academia, industry, government agencies and political leaders, over these past 17 years. I believe the submission is well-informed about the core issues based on this wide experience and my deep immersion in rare disease policy issues for such a significant time.

I hope you find this submission of interest in your work. I am happy to have it made public by the Panel and to answer any further questions you have about it.

Yours sincerely,
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