
**INTELLECTUAL PROPERTY AND PUBLIC HEALTH:
A PATIENT PERSPECTIVE ON CURRENT ISSUES**

A submission to WHO's Second Public Hearing on Public Health Innovation and Intellectual Property on behalf of the Canadian Organization for Rare Disorders (CORD)

The Canadian Organization for Rare Disorders (CORD) supports efforts towards developing a global strategy to address the lack of safe and effective medications for many patients around the world and especially those suffering from diseases and conditions that have been “neglected” by pharmaceutical research and development for a variety of reasons.

In order to ensure optimal care for patients around the world, it is important that:

- A strong research environment be protected;
- Appropriate incentives are provided to ensure research into diseases where it may be difficult for companies to recoup the cost (tropical diseases, rare disorders, etc.);
- Effective strategies for improving treatment access be promoted; and
- Patients be involved in ongoing consultations regarding global health research and development and patent issues.

RECOMMENDATIONS

CORD fears that debate around the issues of patent protection and drug access have become polarised by different stakeholders, with different interests. These issues have become a debate about developed versus developing economies or, more versus less stringent Intellectual Property Regimes. Meanwhile, the interests of the key stakeholders – patients – have been neglected.

Moving forwards, CORD recommends the following actions be taken:

Ensure patient involvement in ongoing discussions around IPP as it relates to medical R&D and healthcare.

- It is critical for patients to engage in these discussions around IPP, and particularly important for patient-based groups to understand the implications of the WHO IGWG draft strategy and plan, so that the needs of all patients are directly reflected and patient-centered interests drive the agenda.
- We urge the WHO and all stakeholders to take significant action to ensure that patients and patient groups are given a central role in future discussions.

Ensure strong incentives for continued R&D and innovation.

- These incentives — along with other focused strategies such as public-private partnerships and similar collaborative arrangements — are essential to the development of new products for patients and for expanded access to better health care in developing nations.

Ensure continued incentives for R&D into rare disorders.

- Free-market incentives provided by the Orphan Drug Act (and similar legislations around the world) have stimulated the development of treatments for rare disorders, for which patients were previously without hope.

Support strategies to improve access to safe treatments.

- Differential pricing for new therapies may be offered to low GDP countries where healthcare systems cannot (or will not) pay. This is feasible because the majority of “innovation investment

incentives” are recouped from sales in the developed markets and these sales represent “marginal” costs of production.

- A key problem with “differential pricing” has been arbitrage, or re-sell of drugs from the lower priced purchasers into the developed country markets, which undercuts the profits to the innovator or the development of a “black market” which undermines the intention to provide drugs to patients in a specific country. Some manufacturers have developed special packaging or formulation to avoid re-sell.

Support strategies and incentives to improve research into neglected diseases.

- Market-based incentives intended to promote research into under-served needs and areas include “pooled advance purchase commitments” for medicines such as vaccines, whereby governments, foundations, and international alliances would commit to purchasing specified quantities of vaccine at contracted prices and then give it to the poorest countries (at affordable prices).
- An alternative route proposed is the Medical Research & Development Treaty (MRDT) whereby countries would pool funds to develop treatments for under-served areas and disorders. The proposal is based on equitable sharing of costs of R&D, provides incentives to invest in useful R&D in areas of need and public interests, and recognizes human rights and goals of all sharing in benefits of scientific achievements. The premise is that government coordinated R&D is fairer and more efficient than market system. However, there has been no consensus and there is no evidence that the proposal would actually generate new therapies.

THE ROLE OF PATIENT GROUPS & CONCERNS WITH THE WHO IGWG PROCESS

CORD is one of many groups that are extremely disappointed that patients and patient groups have not been actively encouraged to participate and offered the necessary support to do this effectively from the outset of this process.

A fundamental premise of patient-centred care – a concept endorsed by the WHO – is that patients and patient groups are involved in all stages of healthcare decision-making, including setting of international policy.

Given the announced timeframe of WHO and the Intergovernmental Working Group on Innovation, Public Health and Intellectual Property, there is literally no time for patient groups to inform themselves and consult with each other regarding many of the issues and potential measures outlined in the strategy.

We are dismayed that the draft plan does not specifically refer to patients or patient groups amongst the key stakeholders. There are many patient groups, including the International Alliance of Patient Organizations (IAPO), that are partnered with WHO and have actively participated in many other health policy issues, at international, national, and regional levels.

Moving forwards, CORD urges the WHO IGWG and all stakeholders to take significant action to ensure that patients and patient groups are actively engaged and are given a central role in future discussions.

INTELLECTUAL PROPERTY ISSUES AND CHALLENGES FOR PATIENTS

INNOVATION AND ACCESS

Access and innovation are inseparable. Without access innovation generates no benefit, without innovation, access leads to limited outcomes.

Patients must have access to the healthcare services warranted by their condition. This requires:

1. Continuing to foster an environment that provides strong incentives to innovate

2. Providing support to those patients that need medicines to access them, and
3. Addressing the issues preventing patients accessing medicines including, basic off-patent medicines in developing countries

Access to Therapies

All patients have a fundamental right to access safe and effective therapies, including medications. To be useful, treatments must be delivered through quality healthcare systems that have appropriate resources and safety measures for diagnosis, surgical and other therapeutic interventions, patient education, and on-going professional and lay support.

The welfare of patients and the public require government-funded programs that address health promotion and the determinants of health (safe water, sanitation, adequate food, physical security, social relations, and meaningful occupation).

Access to Innovative Therapies

There are many challenges for patients in accessing innovative therapies, only one of which is the cost of medicine. Even in developed countries, access to innovative therapies is not uniform or universal and is influenced by many factors, including the ability to pay and access to alternative sources of funding.

There are various strategies used to improve patient access to therapies (both patented and generic) that are used in both developed and developing countries. The cost of therapy may be subsidized by insurers, governments, or philanthropists.

The price charged to some patients may be lowered through bulk pricing (e.g., US Veterans Affairs, Canadian public drug plans, large private insurers or managed care institutions).

Innovation and Rare Disorders

There is probably no greater testament to the importance of strong patent protection to stimulating research into unmet needs than the “orphan drug” legislation, introduced first in the United States in 1983.

The designation of “orphan disease” varies depending on the respective country, ranging from a fairly restrictive ratio of 1:9,090 for Australia (or 2,000 affected individuals) to 1:1,333 (or 200,000 potential patient populations) in the United States. Since these diseases are so rare, affecting a small percentage of the population, there has been little incentive for the pharmaceutical and biotechnology companies to develop orphan drugs. These diseases were also ignored by most academic researchers.

In order to stimulate research and development of medicines as well as devices to diagnose and treat diseases, the United States passed the Orphan Drug Act in January 4, 1983. It took ten years for the second country, Japan, to follow with a similar, albeit less generous, amendment to its drug regulatory and taxation laws. In addition, Australia passed a similar policy in 1999. The policy focused on facilitating market access to drugs that had already received orphan status in the USA and did not provide incentives for Australian R & D. Furthermore, the European Union passed new legislation in 2000 to facilitate the availability and accessibility of orphan drugs. The new legislation granted lower application fees and ten-year market exclusivity, and country-specific research incentives to manufacturers of drugs for rare, life-threatening or serious diseases.

The results of the Orphan Drug Act have exceeded expectations, with over 268 orphan-designated products brought to market in the USA since legislation (as compared to only 34 in the previous decade), over 300 medicines in clinical trials or awaiting approval, and over 1600 orphan designations awarded. In a 20-year review of the Orphan Drug Act, Scientific American concluded that the “Act

certainly has its warts, but in a free-market economy, it is the best model devised so far to ensure that those with rare diseases can get the treatments they so desperately need.”¹

Drug Access in the Developing World

There are many barriers to access to healthcare in developing countries, however the principal barrier is poverty. Poverty affects the availability of food, clean water, hospitals, clinics and healthcare professionals, all of which are key factors in improving health outcomes in poor countries.

There are genuine disparities in global health. Development assistance for health purposes has grown exponentially via new initiatives by the Bill and Melinda Gates Foundation, among others, and new organizations such as the GAVI Alliance and the Global Fund to Fight AIDS, TB and Malaria. These entities have spent billions in developing viable medicine procurement and delivery systems for least-developed countries. Nevertheless, many critics of international patent rules, such as Health Action International, oppose private-public partnerships and drug donations that have been effective in helping to improve health outcomes in developing countries.

SAFETY

Patients have a right to access safe, quality and appropriate services, treatments, preventive care and health promotion services. To this end, the draft strategy must ensure that all medicines, wherever they are developed or produced, must pass through a stringent regulatory framework that guarantees:

1. The quality of the manufacturing process;
2. The security of the supply chain, and
3. High quality information provided to patients, e.g. information to help patients take their medicines correctly and hence safely and to help patients identify counterfeit medicines.

To assure patient safety, medicines must be made available to patients within a healthcare environment that includes appropriate diagnostic, monitoring, and supportive treatment and care

Impact of International Patent Agreements

The Trade-Related Intellectual Property Rights Agreement (TRIPS) was introduced in 1994 and significantly affected the ability of countries with capacity to copy (but not innovate) from copying existing medicines. The standards to be enforced were based on developed countries, resulting in a “worldwide upward harmonization with minimum standards.”

The TRIPS agreement also specified exception to patent rights (Article 30: Other Use Without Authorization) including compulsory licensing for self or 3rd party where “public interest” was concerned. However, compulsory licensing was predominantly to supply a “domestic market” and was not intended for export to countries without sufficient manufacturing capacity.

The Doha Declaration (2003) provided a significant addendum to TRIPS. On the one hand, it acknowledged that intellectual property protection (IPP) was a strong incentive for the development of new drugs. However, it declared that IPR can damage public health through effect on price and thus demanded that manufacturers in developing countries (or any environment) could seize a patent and copy a drug for its own use or for another country without technical capacity. Moreover, the country could determine the grounds for compulsory licensing.

Regulating Compulsory Licences

A key problem for patients is the lack of quality standards (regulatory control) for treatments manufactured under a compulsory licence and imported into developing countries.

¹ Maeder T. The Orphan Drug Backlash. Scientific American, May 2003, 81-87.

- Surveys find that 10 to 20% of drugs in developing countries fail quality control tests.
- Fewer than “1 in 3” developing countries have fully functioning drug regulatory systems, leading to growing number of fake drugs.
- Between 25 - 50% of medicines in developing countries are estimated by WHO to be counterfeit.

In addition to the questionable quality of treatments produced, inefficiencies in procurement, storage, prescribing, and use of drugs are so extensive in many developing countries that consumers benefit from an estimated \$12 worth of treatment for each \$100 spent by the public.

In the case of anti-retroviral medicines (ARVs), the use of sub-standard products promotes resistance to first-line therapies and requires patients to use additional treatments. First-line therapies are the cheapest and most common form of ARVs. The cost of moving patients onto second-line therapies is significant and requires a higher level of care with supportive infrastructure and medical services. Drug resistance is also cumulative. It is estimated that up to 45% of Thailand’s AIDS patients are now drug resistant.

NEGLECTED DISEASES AND “NEEDS-BASED” R&D

A criticism of the current global environment for pharmaceutical research and development is that international drug research has failed developing countries by ignoring tropical diseases such as malaria. While tropical diseases deserve more attention, it is important to note that universal diseases such as cancer, diabetes, and cardiovascular disorders cause more morbidity and mortality in developing countries than tropical diseases.

The research-based pharmaceutical industry is responsible for the development of all of the most effective drugs for treating cancer and cardiovascular diseases. The research-based pharmaceutical industry is also responsible for the development of all 22 of the currently available anti-retroviral treatments for HIV/AIDS.

RATIONALE FOR PROTECTING INTELLECTUAL PROPERTY

The challenge for policy makers is to implement systems that encourage continued development of new therapies (for treated or unmet needs) while assuring therapies are available to patients to maximize health benefits.

A strong patent system works well to stimulate development by providing incentives to developers in the form of a “limited-term monopoly.” whereby profits are returned to the developers through the sale of inventions (medical products). Most drugs used today—whether they are “brand-name” medicines or generic copies—are the products of a system that has encouraged innovation by protecting intellectual property through patents.

ALTERNATIVE ROUTES (GENERIC COPYING AND COMPULSORY LICENSING)

Some countries (e.g., India, Brazil) have had very “loose” patent systems, which has allowed indigenous manufacturers to copy patented medicines and distribute these to their own patients. Developing countries rely on rules of compulsory licensing, for example, to hand over a patent to a local manufacturer by declaring that there’s a public health need or that insufficient product is available to the patient population.

As discussed above, a key problem for patients is the lack of quality standards (regulatory control) for treatments imported into developing countries.

In addition to the questionable quality of treatments produced, inefficiencies in procurement, storage, prescribing, and use of drugs is so extensive in many developing countries that consumers benefit from an estimated \$12 worth of treatment for each \$100 spent by the public.