7. Access to effective treatments for cancers and rare and orphan diseases and market transparency

Contents

In focus

The Secretariat advises:

> At the recommendation of the Officers of the Executive Board in 2019, and pursuant to the following resolutions, the Director-General has prepared EB148/9 which reports (Part A) on progress in implementing WHA70.12 (2017) regarding access to health products for rare and orphan diseases, and (Part B) on implementing the market transparency resolution WHA72.8 (2019).
>
> The Board is invited to note the progress made and to provide further guidance on optimizing access to cell- and gene-based therapeutics and other health products for rare and orphan diseases.

Background

See the commentary by Thiru on KEI OnLine (21 Dec 2020) here for the background into Part A of EB148/9. Thiru notes the absence from EB148/9 of any response to the 2019 request by South Africa for WHO to "discuss the role of the public sector and charities in funding research for new cell and gene therapies, and measures to promote more transparency of the licensing of intellectual property rights from public sector research, and concrete measures in licenses to address the objective of universal access."

Thiru notes that EB148/9 does not fully address the issues raised by Peru in relation to the availability and affordability of medicines for rare and orphan diseases.

See also WHO informal consultation (3 December 2020) addressed concerns on price transparency and shortages by Thiru of KEI, posted on 12 Jan 2021

Recent GB discussions of medicines, including access

Secretariat topic page for Medicines

For more on cell and gene based therapies see

- Dunbar et al (2018) Gene therapy comes of age;
- Shah et al (2018) Multi-targeted CAR-T Cell Therapies for B-Cell Malignancies
- Jin et al (2019) Stem cell therapies for acute spinal cord injury in humans: a review

PHM Comment (Draft, work in progress)

Part A. Access to safe, effective, quality-assured and affordable health products for cancer and rare and orphan diseases

The paper sets out the problem clearly: *'Despite recent advances, access to safe, appropriate, effective and quality-assured health products remains a global concern'*. The paper mentions a range of barriers to access, focusing largely on domestic health system issues (but not mentioning intellectual property barriers).

The paper then elaborates on some particular challenges including vaccines (hepatitis B and HPV), rare diseases with and without treatments, particularly expensive therapies (including cell therapies, gene therapies and cell-based gene therapies), medical devices, diagnostics and assistive products.

The paper then describes what the WHO Secretariat is doing in a range of related fields. The paper does not provide a comprehensive analysis of the barriers to access nor the range of structural reforms which might be needed to address these barriers. The focus is on what the Secretariat is doing. Even under the heading, 'The way forward', the focus remains on what the Secretariat will do.

The exception is para 22 which calls for *'more collaborative work … to shape research, innovation and development with a view to encouraging the development of affordable solutions for low- and middle-income primary health care settings for the management of cancer, other noncommunicable diseases such as diabetes, and rare and orphan diseases'*.

The field which this paper addresses is vast; a wide variety of technologies including vaccines, medicines, medical devices, in vitro diagnostics and assistive products all with complex value chains from conception to utlisation. Assuring affordable equitable access requires policy interventions and structural reforms at all stages in these value chains including research, patenting and IP protection, production, pricing, regulation, health systems, procurement, and health care financing.

The configuration of these value chains, through which access is shaped, varies widely across different products, institutional settings and countries. Nevertheless we can identify, in general, some of the main barriers and possible solutions at different points across the chain.

Research, research capacity and research funding

More research funding is required, with funding redistributed to conduct research into developing interventions to neglected diseases which disproportionately impact LMICs and therefore hold smaller financial incentives for pharmaceutical companies. An R&D binding convention proposed in 2013 to ensure sustainable financing and equitable access would serve as an effective mechanism to address this issue and should be discussed by MS and WHO further (read more here). This should also be complemented by the R&D Observatory as per the original proposal of the CEWG.

Research funding should be delinked from IP protection and monopoly pricing. This can be implemented by research funding sponsors, including public research councils whereby a funding requirement would be for the recipient to have a global access plan, for example to retain their IP rights or pursue non-exclusive licensing.

Development partnerships and technology transfer can serve as mechanisms to facilitate the strengthening of research and innovation capacity in LMICs. This can also serve to increase clinical trials conducted in LMICs which are often neglected even in treatments for diseases with high burden.

Patenting and IP

Open innovation refers to the sharing of patents, design, copyright and technical know-how. It is necessary to allow disseminated local production and continued product development. This will facilitate improved safety and efficacy, cheaper production and lower prices and therefore should be a key recommendation to increase access.

Pricing

By increasing transparency over pharmaceutical pricing thereby showing how much each country pays, it would allow comparison and strengthen countries negotiating leverage, facilitating fairer pricing and better access. This would particularly benefit countries with less negotiating power, and would protect against secret inequitable pricing strategies.

Full use of TRIPS flexibilities would allow more local production of pharmaceuticals, building longer term public sector production capacity thereby circumventing monopoly pricing at a global level and associated price gouging.

Transparency regarding development costs

Pharma and its advocates claim that high prices are necessary to allow for the recovery of huge investments in R&D. However, the costs of drug development are far from transparent and the actual costs borne by Big Pharma are even less so.

Over the last two decades the underlying business model of pharmaceutical development has evolved into a pattern referred to as financialisation. In accordance with this model, the basic research upon which all therapeutics are based is largely publicly funded. Likewise the early development work, more deliberately directed to clinically useful outcomes, generally receives a significant amount of public funding. As a particular line of work shows promise the researchers are encouraged to start up dedicated development enterprises, often with the support of venture capital investors. Some of these startups will fail and the cost of failure will be borne by the principals and investors. However, those startups which show more promise are now acquired by the large pharmaceutical corporations, including their patents, designs and knowhow. Acquisition by Big Pharma adds to stock value and encourages further outside investment. From this point share price manipulation (including public relations initiatives, stock buybacks, shareholder payouts, and executive bonuses) plays a key role in pharma strategy. Pharmaceutical companies spend more on mergers and acquisitions than in-house 'R&D'.

The argument that high prices are necessary to recoup R&D costs is largely smoke and mirrors with a complete lack of transparency. While the costs of basic research and early development work (including unsuccessful startups) may be significant, under the financialisation paradigm, these costs are not borne by Big Pharma. Rather Big Pharma can wait until the more promising startups emerge, buy them cheaply and then inflate their market value.

The development of health products is expensive and under the logic of the patent system it is appropriate to price the final product at a level which covers *those costs of R&D which were borne by the vendor*. However, while financing of R&D and the distribution of costs remain so untransparent there are firm grounds for concern that market prices set under the protection of patent protection may be too high.

Improving access to expensive therapeutics could be greatly enhanced if unreasonable prices were exposed through greater transparency regarding financing and costs.

Marketing

The dominant narrative justify high prices is to allow recovery of research and development investments by pharmaceutical companies. Yet, companies often spend more on marketing than on R&D. Tighter regulation of such activities would reduce a key 'cost'. One route to achieve this would be for governments to adequately fund independent expert prescribing advice, including through social marketing and academic detailing and directed to both providers and consumers.

Health system strengthening

PHM appreciates the emphasis on the crucial role of primary health care (PHC) in facilitating affordable access to various health products, including for rare and orphan diseases.

Improving access requires a focus on health systems strengthening as well as attention to the development and marketing of pharmaceuticals and other health care products. Key areas to address are pharmaceutical procurement; pooled procurement in which a consortium of

hospitals, regions or at national level jointly purchase pharmaceuticals mitigates price gouging and permits stronger negotiating power and thus lower prices.

The public sector should provide healthcare without user charges. The World Bank's model of UHC as comprising mixed service delivery financed through competitive health insurance markets is a recipe for increased costs both for government and for health insurance purchasers.

Regulation system strengthening

Regulatory capacity is critical if the safety and efficacy of novel therapies are to be assured and if their utilisation is appropriately directed.

The harmonisation of regulatory standards has benefits for both the manufacturers and for governments but standard setting needs to be protected from corporate influence variously directed to preventing competition and allowing scope for price inflation.

Ensuring safety and efficacy as well as enhancing access depends on the probity and accountability of the regulators, including standard setting institutions.

Macroeconomic reform

Reform is also needed to address the revenue limitations facing the governments of low and middle income countries. These include low tax small government regimes driven by the international financial institutions (and by investor extortion); the choking of export opportunities in a global economy facing a growing overhang of productive capacity over purchasing capacity; and the loss of social solidarity associated with widening economic inequality.

Key reforms to enhance access to medicines in L&MICs must include addressing the fiscal constraints they face, starting with global tax justice and fair trade.

Part B. Market transparency

EB148/9 provides a useful review of a range of initiatives underway directed to promoting market transparency, including price, patent status, and research, development and production costs.

A key set of initiatives are directed to addressing the legal barriers to publication of prices, including through tying price transparency to marketing approval and legislative reform regarding 'commercial in confidence'.

These initiatives are commendable. We look forward to hearing updates regarding MS implementation of transparency so that it becomes an embedded legislated norm.

Notes of discussion