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Expanding access to effective treatments for cancer and rare and orphan diseases, including medicines, vaccines, medical devices, diagnostics, assistive products, cell- and gene-based therapies and other health technologies; and improving the transparency of markets for medicines, vaccines, and other health products

Report by the Director-General

1. This report presents progress made in implementing resolution WHA70.12 (2017) on cancer prevention and control in the context of an integrated approach, on and access to health products for rare and orphan diseases[1] (part A) and in implementing resolution WHA72.8 (2019) on improving the transparency of markets for medicines, vaccines, and other health products (part B).

A. ACCESS TO SAFE, EFFECTIVE, QUALITY-ASSURED AND AFFORDABLE HEALTH PRODUCTS FOR CANCER AND RARE AND ORPHAN DISEASES

Background

2. Safe, effective, quality-assured and affordable vaccines, medicines, medical devices, in vitro diagnostics and assistive products are necessary for the prevention, diagnosis and treatment of cancer and other noncommunicable diseases such as diabetes, and rare and orphan diseases, as well as the monitoring, rehabilitation and palliative care of individuals with these conditions. This need is recognized in resolutions, strategies and reports.[2]

3. Despite recent advances, access to safe, appropriate, effective and quality-assured health products remains a global concern. For example, only 1 in 10 people who need assistive products has access to

[1] Diseases affecting small numbers of patients, including diseases of genetic origin, infrequent forms of cancer, autoimmune diseases, toxic and/or infectious diseases, and congenital deformities.

[2] WHA73.2 (2020) on the global strategy to accelerate the elimination of cervical cancer as a public health problem and its associated goals and targets for the period 2020–2030; WHA71.8 (2018) on improving access to assistive technology; WHA67.20 (2014), on regulatory system strengthening for medical products, WHA63.22 (2010) on human organ and tissue transplantation and WHA60.29 (2007) on health technologies; and document A72/17, Access to medicines and vaccines.

them.[1] Even when medicines are available, associated medical devices are often not. For example, very few countries provide test strips as part of diabetes care programmes to help to monitor blood glucose concentration in individuals who require insulin.

4. Challenges exist throughout the health system, especially in low-income countries. They range from inadequate investment in research and development, lack of effective policies for selecting health products, inadequate financing and expenditure management, weak regulatory capacity, poor infrastructure and insufficient resources for procurement and supply chain management, to inappropriate prescribing and irrational use of health products.

5. Because many patients pay for health products through out-of-pocket expenses, even low-cost products may be unaffordable. Chronic conditions require long-term care, placing a greater burden on patients and governments. Rare and orphan diseases affect a small number of patients and their management presents specific challenges, including the need for complex and specialized care. These diseases may not be considered a priority in universal health coverage packages and may thus be left out of public procurement and reimbursement.

Particular challenges

6. Hepatitis B vaccine prevents hepatitis B-related cancers and other diseases. In 2019, 191 of 194 Member States had the three-dose series in their national vaccination schedule and 85% of the world's infants received three doses of the vaccine. However, only 43% of newborns received a birth dose, which prevents mother-to-child transmission of hepatitis B virus. Only 129 Member States follow the WHO recommendation of vaccinating at birth. In the African Region, where prevalence rates of hepatitis B in the population are high, only 28% of Member States provide the birth dose.

7. Human papillomavirus vaccine is an effective means of preventing cervical cancer. The number of countries introducing the vaccine has increased in recent years, but low- and lower-middle-income countries still lag behind. High prices of the vaccine and a limited number of suppliers pose barriers to access in some countries. Other challenges include the delivery strategy, the quality of the plan to introduce the vaccine into the national schedule, insufficient demand creation, and a lack of confidence in the vaccine, sometimes based on rumours. Global supply shortages threaten introduction in low- and middle-income countries.

8. There are no treatments for many rare and orphan diseases. When a treatment exists, its availability may depend on domestic legislation and regulations, including national orphan drug policies, orphan drug designations and marketing authorizations.

9. Cell therapies, gene therapies and cell-based gene therapies have the potential to meet the medical needs of individuals with certain cancers and rare and orphan diseases. Cell therapies, gene therapies and cell-based gene therapies vary in nature, and the relevant regulatory framework and evaluations are not harmonized or even in place. The high prices of these products and patent barriers limit access to a few high-income countries: chimeric antigen receptor T-cell therapy, for example, is priced in the

[1] WHO and World Bank. World report on disability. Geneva: World Health Organization; 2011 (https://apps.who.int/iris/handle/10665/44575, accessed 27 October 2020), and WHO, UNESCO, ILO and International Disability Development Consortium. Community-based rehabilitation: CBR guidelines. Health component. Geneva: World Health Organization; 2010 (https://apps.who.int/iris/handle/10665/44405, accessed 27 October 2020).

hundreds of thousands of US dollars and gene therapy for spinal muscular atrophy is priced in the millions of US dollars.

10. Particular challenges related to medical devices, in vitro diagnostics and assistive products include a lack of user- and context-appropriate technologies. There is little research and development of cost-effective, user- and context-appropriate design, and advances are mostly geared towards high-income countries. There is a lack of support for maintenance, repairs, access to reagents, consumables and spare parts, and a shortage of health workforce capacity. Access to medical devices is complicated by the absence of a common naming system to identify more than 7000 different types.

11. Most medical equipment for diagnosis and treatment of cancer is found in specialized hospitals. Assistive product services are often stand-alone programmes and not integrated across all levels of care.

Progress

12. The Secretariat has provided support to countries for the implementation and monitoring of hepatitis B and human papillomavirus vaccination programmes and for improving procurement practices. It is enabling pricing transparency through the Market Information for Access to Vaccines initiative.[1] The WHO Strategic Advisory Group of Experts on Immunization has developed recommendations to deal with supply shortages and reviewed evidence for more efficient use of the vaccines. WHO started a global access dialogue on human papillomavirus vaccines to facilitate fair access to available doses. Strategies to eliminate viral hepatitis and cervical cancer are expected to speed up progress.

13. The Secretariat has developed standards to ensure the quality, safety and efficacy of human papillomavirus vaccines, and provided support for manufacturers, regulators and others in order to facilitate regulatory convergence and enable WHO prequalification. The safety of human papillomavirus vaccines is continuously monitored and reviews have shown an excellent safety profile. The Secretariat has carried out post-introduction evaluations of the vaccines in countries and provided guidance on programme improvement.

14. A report on the pricing of cancer medicines[2] presents options for enhancing the affordability and accessibility of cancer medicines, including through price transparency and by realigning incentives for research on cancers that affect fewer people. The Secretariat has engaged in policy discussions, including at the 2019 Fair Pricing Forum. The WHO Report on Cancer provides guidance on health system considerations.[3] The Secretariat has developed clinical guidelines, to help to support Member States, and has developed a series of accompanying tools. WHO initiatives on cancer include the WHO Global Initiative for Childhood Cancer.

15. Medicines for cancer and rare and orphan diseases on the WHO Model List of Essential Medicines represent a small proportion of the total number of medicines approved and marketed for cancer and

[1] See https://www.who.int/immunization/programmes_systems/procurement/v3p/platform/en/ (accessed 15 October 2020).

[2] Technical report: pricing of cancer medicines and its impacts. Geneva: World Health Organization; 2018 (https://apps.who.int/iris/handle/10665/277190, accessed 15 October 2020).

[3] WHO report on cancer: setting priorities, investing wisely and providing care for all. Geneva: World Health Organization; 2020 (https://apps.who.int/iris/handle/10665/330745, accessed 15 October 2020).

rare and orphan diseases worldwide. The list helps Member States to prioritize medicines for public financing, procurement and reimbursement.

16. In 2018, WHO launched a pilot for prequalification of biotherapeutics to treat cancer. Twelve biosimilar products and seven products for neglected tropical diseases have been prequalified. A risk assessment procedure facilitates access to additional products. WHO has defined norms and standards for ensuring safe and effective cell and gene therapy products and adopted reference materials for measurement standards. It assigns International Nonproprietary Names, including for cell therapies, gene therapies and cell-based gene therapies (such as chimeric antigen receptor T-cell therapies and gene replacement therapies for rare genetic disorders). The International Nonproprietary Names are published together with harmonized definitions in the journal *WHO Drug Information*. These are essential for clear identification, prescription and use of complex substances.

17. A number of gene therapy products have been approved, such as chimeric antigen receptor T-cell products for B-cell tumours and an oncolytic immunotherapy for the treatment of metastatic melanoma. This reflects the advances in research and development and in regulatory pathways.

18. The Secretariat is collaborating with Rare Diseases International to shape international policy and strengthen the capacity of health systems to address the challenges related to rare diseases. Activities focus on harmonizing the way in which rare diseases are defined internationally and on laying the groundwork for a global network of centres of excellence for rare diseases.

19. The Secretariat works with the Worldwide Network for Blood and Marrow Transplantation to raise awareness of transplantation availability and access, build regulatory capacity, enhance governance to tackle illegal and unethical practices, increase knowledge, support innovation and research new products.

20. The Secretariat recently produced guidance for increased access to medical devices includes the following: the WHO Model List of Essential In Vitro Diagnostics, which contains tests for cancer and diabetes; the WHO list of priority medical devices for cancer management; and WHO technical guidance and specifications for medical devices to screen and treat precancerous lesions in the prevention of cervical cancer.

21. The Secretariat recently produced guidance for increased access to assistive products includes the Priority Assistive Products list and the rapid Assistive Technology Assessment toolkit. The Secretariat provides support to Member States in their efforts to measure the demand for, and barriers to accessing, assistive technology, and to regulate, finance, procure and provide assistive technology. Country capacity assessments using the WHO toolkit are conducted jointly by WHO, the Global Disability Innovation Hub and the Clinton Health Access Initiatives.

The way forward

22. More collaborative work is needed 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.

23. The Secretariat will continue to provide support to Member States action to select essential and priority health products by developing model lists of such products and related technical specifications for priority products to guide procurement. It will continue to work on the standardization of

nomenclature and naming for medical devices. It is also developing an online training package to ensure that the workforce has the competencies needed to provide a range of assistive products.

24. WHO will continue its work on regulatory system strengthening, including approaches for harmonization and recognition, to ensure the safety, efficacy and quality-assurance of health products, and to bolster the capacity of Member States to regulate medical devices, in vitro diagnostics and assistive products.

25. The Secretariat will provide guidance and training to ensure appropriate use of health products for cancer, diabetes, and rare and orphan diseases, and promote the training of biomedical engineers, laboratory scientists and pathologists.

B. IMPROVING THE TRANSPARENCY OF MARKETS FOR MEDICINES, VACCINES, AND OTHER HEALTH PRODUCTS

26. In describing progress made to date and next steps regarding implementation of resolution WHA72.8 (2019), this part of the report refers to relevant sections of the global strategy and plan of action on public health, innovation and intellectual property, and the draft road map for access to medicines, vaccines and other health products, 2019–2023.

27. Some countries have made political commitments to improve market transparency. The European Region plans to convene a meeting in 2021 for its Member States and relevant stakeholders to discuss approaches to improving transparency and affordability of high-cost innovative medicines. The Eastern Mediterranean Region is considering a proposal to establish mechanisms that improve collaboration and information exchange between countries on prices of medicines and vaccines. The Secretariat has raised awareness of this issue via the Pharmaceutical Pricing and Reimbursement Information network. It continues to explore the feasibility and potential value of an international data platform and forums for sharing of information on prices and pricing approaches.

28. Work is being done in the European Region and the Region of the Americas to explore the legislative barriers to transparency, given the acknowledged impossibility of promoting price and pricing transparency under the laws and (confidential) commercial agreements in many jurisdictions.

29. WHO has published a revised guideline on country pharmaceutical pricing policies. The Secretariat is working with the Government of Argentina to prepare the agenda of the 2021 Fair Pricing Forum.

30. Evidence on the potential impact of promoting the transparency of prices and pricing of pharmaceutical products from comparative studies remains limited. Ongoing work on Sustainable Development Goal indicator 3.b.3, on access to medicines, will provide insight on affordability. Member States have used the WHO MedMon mobile application and data analytics platform to monitor prices in the public and private sectors, and to assess adherence to pricing policies.[1] Many Member States reported having presented medicine price information in the public domain as part of a regular procedure.

31. In line with the global strategy and plan of action on public health, innovation and intellectual property, WHO continues to request and strongly promote the further development of patent status

[1] MedMon – WHO Essential Medicines and Health Products Price and Availability Monitoring Mobile Application (https://www.who.int/medicines/areas/policy/monitoring/empmedmon/en/, accessed 15 October 2020).

information and licensing databases for health products and facilitates greater access to such information by public health actors, in particular, procurement agencies. MedsPaL, a publicly available patents and licenses database established by the Medicines Patent Pool, contains information on the patent status of medicines for treatment of HIV, hepatitis C and tuberculosis, and other patented essential medicines, in low- and middle-income countries.

32. WHO is finalizing its third global report on access to hepatitis C treatment. This report, which will update the information in the previous reports published in 2016 and 2018, will include information on hepatitis C diagnostics. With a focus on selected countries with a high burden of hepatitis C virus infection, the report will highlight recent achievements and provide updates on the diverse and complex landscape of national strategies and conditions of access to diagnostic and pharmaceutical products, including product pricing and availability of patent status information, which together shape a national hepatitis response.

The way forward

33. Despite the progress made, much remains to be done. The 2021 Fair Pricing Forum will be an important milestone at which to evaluate lessons learned and to reaffirm the commitment of Member States and all relevant stakeholders to health product affordability and transparency of prices and costs. The Secretariat will continue to provide technical support and guidance to Member States in their efforts to achieve and monitor transparency of markets for health products.

34. Renewed political commitment remains essential if countries are to make progress towards transparency of markets for health products. Successful collaboration requires increased efforts to mobilize resources and strengthen capacity.

ACTION BY THE EXECUTIVE BOARD

35. The Board is invited to note the report and provide guidance on access to cell- and gene-based therapies and other health technologies for rare and orphan diseases.

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