EVIPNet CHILE

Report on Policy Dialogue

Financing Options for rare diseases in Chile

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EVIPNet-Chile

EVIPNet stands for Evidence Informed Policy Network. The network, sponsored by the World Health Organization (WHO), uses a systems approach to strengthen health systems by improving the links between policy and research evidence for health, especially in low and middle income (LMIC) countries.

To achieve this, country teams championed by health authorities are organized with key stakeholders, such as policy makers, researchers and representatives from other sectors. These teams identify and address relevant policy issues where a perceived need of using research evidence to inform decisions about health policy has been identified. EVIPNet therefore includes components relevant to research and to development and is expected to help strengthen health systems

EVIPNet Chile began its activities on the first semester of 2011. The team is composed by policy makers from the Ministry of Health and the National Health Fund (FONASA) and researchers from the School of Public Health of the University of Chile and from the Health Policy and Systems Research Unit at the Pontificia Universidad Católica de Chile.

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Conflict of interest

The authors declare that they have no professional or commercial interests relevant to the issue discussed in the deliberative dialogue .

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SUMMARY OF THE DIALOGUE

A rare disease is a disease that occurs infrequently or rarely in the general population. In order to be considered as rare, each specific disease cannot affect more than a limited number of people out of the whole population, defined in Chile as 0.18 in 10,000 citizens.

Ultra-orphan drugs are medicines used to treat exceptionally rare diseases that are chronically debilitating or life-threatening. Low patient numbers make it difficult for pharmaceutical companies to recover research and development costs, and consequently these medicines are generally expensive on a per patient basis.

Drawing on the input from the policy brief, their own knowledge and experiences, and the insights from the deliberations, a number of participants in the dialogue indicated that:

- Cost-effectiveness is a mayor criteria used in Chile to define the health problems that are funded by the public health system
- Currently, the judicial courts have treated a number of cases concerning the right of rare disease patients to health care. The problem is the possible effects of certain court decisions on the rules that guide the health system
- Special funding status for orphan drugs will avoid dealing with the problem of financing interventions that are cost effective compared with other health interventions that are not currently funded.
- Right now in the Chilean political context the best alternative for funding rare diseases is mandatory private health insurance
- The mandatory private health insurance option is a more transparent method of financing than tax funding, since households contribute directly, and receive a specified benefit package.
- Regardless of how funds are raised, decisions must be made about how to pool the funds, and how to use them to purchase services

BACKGROUND TO THE DIALOGUE

A rare disease is a disease that occurs infrequently or rarely in the general population. In order to be considered as rare, each specific disease cannot affect more than a limited number of people out of the whole population, defined in Chile as 0.18 in 10,000 citizens (Minister of Health draft of the law on rare diseases). This figure can also be expressed as 18 patients with rare diseases out of 1 million citizens. While 0.18 out of 10,000 seems very few, in a total population of 17 million citizens this could mean as many as 306 individuals for each rare disease. It is important to underline that the number of patients with rare disease varies considerably from disease to disease, and that most people represented by the statistics in this field suffer from even rarer diseases, affecting only one in 100,000 people or less. Some rare diseases do only affect a couple of dozens patients. These very rare diseases make patients and their families particularly isolated and vulnerable.

Ultra-orphan drugs are medicines used to treat exceptionally rare diseases that are chronically debilitating or life-threatening. Low patient numbers make it difficult for pharmaceutical companies to recover research and development costs, and consequently these medicines are generally expensive on a per patient basis. As ultra-orphan drugs usually are not cost-effective, and cost-effectiveness is a mayor criteria used in Chile to define the health problems that are funded by the public health system, the Ministry of Health requested EVIPNET Chile a policy brief on financing options for ultra-orphan drugs.

The policy brief was prepared by a team comprised by policy makers from the Ministry of Health and the National Health Fund (FONASA) and researchers from the School of Public Health of the University of Chile and from the Health Policy and Systems Research Unit at the Pontificia Universidad Católica de Chile. It employs a user-friendly format and offers evidence informed policy options on financing alternatives for ultra-orphan drugs.

EVIPNET Chile held a policy dialogue with policy decision makers from the Ministry of Health, Ministry of Finance, National Health Fund (FONASA), and Ministry General Secretariat of the Presidency to discuss their views on the problem, on options for addressing the issue; and key implementation considerations. The dialogue was held on October 3, 2011 at the main building of the Ministry of Health.

Key features of the dialogue were:

- it addressed an issue currently being faced in Chile;
- it focused on different features of the problem, options for addressing the policy issue; and key implementation considerations;

- it was informed by a pre-circulated draft policy brief prepared by EVIPNet Chile that contemplated both global and local research evidence about the problem, options for addressing the problem, and key implementation considerations;
- it brought together parties from the Ministry of Health, Ministry of Finance, National Health Fund and Ministry General Secretariat of the Presidency who would be involved in future decisions related to the issue;
- it included a facilitator to assist with the deliberations;
- it allowed for frank, off-the-record deliberations; and
- it did not aim for consensus.

SUMMARIES OF THE DELIBERATIONS

DELIBERATION ABOUT THE PROBLEM

Dialogue participants noted a number of key features of the problem:

- Rare diseases, 80% being genetic in origin, are defined as those diseases which affect not more than 0, 18 per 10 000 persons in Chile. The limited number of patients accounts for a current low level of medical expertise, even though these diseases can lead to the death or disability of the people affected.

- Most of the laws on rare diseases in different countries focus on marketing and production and not in access to ultra-orphan drugs.

- Raising funds through mandatory prepayment provides the most efficient and equitable path towards universal coverage of rare diseases.

- Cost effectiveness plays an important part in current decisions about the funding of health technologies in Chile. Drugs for rare disease (orphan drugs) are often expensive and, by definition, will benefit only small numbers of patients.

- Orphan drugs tend to be expensive for two reasons. Firstly, development and production costs need to be offset in low volume sales, and, secondly, the monopoly position of manufacturers permits large profit margins.

- While orphan drugs were rare, the Chilean healthcare system was able to deal with them in an ad hoc manner. But there are now many orphan drugs and ultra-orphan

drugs approved by developed countries regulatory agencies. The problem is thus likely to become increasingly common.

- The justification for special status for rare diseases rests on the question: should we value the health gain to two individuals differently because one individual has a common disorder and the other has a rare disorder? Is there a sustainable reason why the cost effectiveness of drugs for rare diseases should be judged differently from that of other healthcare technologies?

- Currently, the judicial courts have treated a number of cases concerning the right of patients with rare disease to health care. The problem is the possible effects of certain court decisions on the rules that guide the health system.

- Based on a constitutional mandate that Chileans are entitled to health, government should undertake concrete measures to ensure universal access to care for patients with rare disease.

- Special funding status for orphan drugs will avoid dealing with the problem of financing interventions that are not cost effective compared with other health interventions that are not financed.

DELIBERATION ABOUT POLICY OPTIONS

Dialogue participants discussed some of the options that were summarized in the draft policy brief:

Mandatory private health insurance

- Participants pointed out that Chile must make decisions about how best to raise sufficient funds for health, how to pool them together to spread the financial risks of ill health, and how to ensure they are used effectively, efficiently, and equitably. Countries make different decisions in each of these areas, so the resulting financing systems vary in areas such as the mix between taxes and insurance, and between public and private funding and provision. Right now in the Chilean political context the best alternative for funding rare diseases is mandatory private health insurance.

- Participants noted that this option is a more transparent method of financing than tax funding, since households contribute directly, and receive a specified benefit package.

- Participants highlighted that one of the issues that will have to be addressed is whether contributions will vary according to income. Important decisions need to be made about how to raise funds equitably and how to ensure equity in access.

- Participants emphasized that regardless of how funds are raised, decisions must be made about how to pool the funds, and how to use them to purchase services.

Tax Funding

- Participants mentioned that tax-based financing is often associated with greater efficiency in revenue collection, since the funds flow directly from the Ministry of Finance to the Ministry of Health. However, the mandatory private health insurance option may be a more transparent method of financing, since households contribute directly and receive a specified benefit package.

- Regarding equity considerations, participants stated that it is not possible to affirm that one method always performs better than the other. In tax-based systems, equity in financing depends on the relative progressivity of the tax system. In mandatory private insurance, the issue is whether contributions are identical for all enrollees, or whether they vary according to income.

DELIBERATION ABOUT IMPLEMENTATION CONSIDERATIONS

Challenges mentioned by participants:

- How to decide on what orphan drugs are going to be covered by mandatory health insurance?

- How much will households pay to obtain health coverage for orphan drugs?

- How much is going to be paid out-of-pocket?

- Since insurance will pay for ultra-orphan drugs that cost more than a certain amount of money? What mechanisms need to be implemented to assure that those coverage limits don´t constitute negative incentives for the pharmaceutical laboratories?
- How much government subsidies for people that cannot contribute?
- How to encourage cost containment strategies for health insurance companies? What incentives need to be employed to assure that increments in costs are not just passed on to the insurance premium?
- What methods for purchasing orphan drugs can be used to warrant the highest value for money? Price of orphan drugs is the major issue.
- How to manage the conflicts of interest in a setting where there are not many physicians with expertise in rare diseases? Most of those physicians have some link with pharmaceutical laboratories that produce orphan drugs.
- To design the insurance plan and the provider network and the need to periodically review the orphan drugs being covered by mandatory health insurance.

NEXT STEPS

A new version of the policy brief will be sent to the Ministry of Health in the next few weeks. The final version will have been enriched by the policy dialogue.

The most important issue is that Chile's government is preparing legislation that will introduce a new financing mechanism for ultra-orphan drugs. The bill, which could be presented to Congress in the next few months, will in some extent draw on knowledge gained from the policy brief and the policy dialogue.

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