AIM contribution to the OECD online consultation on Sustainable access to innovative therapies

1. Reflecting on the last 5-10 years, what do you think have been the major changes affecting access to medicines?

-Prices of innovative pharmaceutical products have increased, especially drugs that come to the market with a reduced target population (specific indication, rare diseases, orphan drugs), and biologicals and advanced therapy medicinal products. These increases have let led to reduction of access to medicines and threatens long term sustainability of healthcare systems.

-Innovative products that have been granted access to the market have had in many cases a too limited added therapeutic value compared to existing alternatives.

- Investigations are largely influenced by the study sponsor who control trial design, data collection/analysis and writes or pays for the publications. Clinical data are not accessible to outside experts. This induces a high risk of bias (e.g. cf. publications by Professor Even and Michel de Longeril on statins).

-After market access has been granted (with a trend of fast market access to specific drugs with limited or no phase 3 clinical trial data at all), healthcare payers, have very limited possibilities (due to time pressure and limited availability of efficacy data) to come to a balanced conclusion about reimbursement and price.

-The world of pharmaceuticals has become more complex, but pharmaceutical care has not developed accordingly. Too many citizens receive the wrong medicinal products or use them in a suboptimal way. Health literacy of users of pharmaceuticals has to be improved to increase correct use and adherence to treatment. Industry, prescribers, wholesalers, payers and pharmacists have a joint responsibility to ensure appropriate use (in particular for patients using many different interacting products simultaneously), including increase of uptake of use of generics and biosimilars.

-In an attempt to bring more equity amongst different treatments and to reward added value instead of mere costs of drugs (research, production, marketing and profit) the traditional “cost plus” method for the setting of prices has been replaced by a new methodology rewarding additional amount of time –life years gained (eventually adapted to the quality of life) compared to other treatments: the broadly used ICER/QALY. In the absence of a clear expression of the societal willingness to pay for this additional life years, the use of a threshold rapidly developed everywhere and led to a rapid increase in all the prices to the level of this threshold, not reflecting anymore any willingness to pay but a mere limit of what the market can bear (cf. US senate investigation against Gilead)..

2. What are the top three (3) issues that must be addressed to ensure access to innovative medicines while maintaining financial sustainability of health systems?

a) Lack of Transparency: The transparency of the sector will have to be increased:

-Transparency regarding patient data in clinical trials – all relevant clinical studies leading to a homologation of an active substance should have the obligation to be accessible in detail for outside reviewers.

-Transparency of the costs of R&D. As R&D costs are used as justifier for prices of pharmaceuticals, better insight in those costs is key.

-Transparency of prices of pharmaceutical products, to be able to have a debate about those prices.

b) At international level, payers and regulators should increase collaboration, aimed at joint negotiation and procurement. Collaborating purchasers have a stronger position during negotiations and can contribute to a fairer price.

c) The society should indicate priorities in drug development, what treatment is needed and how much it wants to collectively pay for that drug that alleviates the unmet need. Do they want to pay for expensive drugs with little added benefits but high return on investment for the pharmaceutical companies or would they prefer to invest the money on other types of health care? This implies a need for more symmetrical information (see above).

3. Why do you think there are issues in ensuring access to innovative medicines while maintaining financial sustainability of health systems?

As mentioned above, there are several trends that threaten the financial sustainability of healthcare systems. In the field of innovative medicines, similar to other parts of the healthcare sector, technological developments increase health but similarly (or probably more significantly) healthcare costs. The ageing of society leads to an increase in healthcare and long-term care spending.

The healthcare sector is different from many traditional markets, with the purchaser and the user being different entities. This market, which is in many countries organized on the basis of solidarity, can only continue to be sustainable if prices of pharmaceuticals are reasonable and used appropriately. Unfair, secret and high prices and inefficient or ineffective use of pharmaceuticals will undermine the possibility to finance the sector (based for a large extent on solidarity principles). Individual patients, doctors and companies have only a limited incentive to maintain the financial sustainability of health systems.

Besides the financial sustainability, the danger of the lack of transparency and lack of evidence are wrong decisions to homologate an early admission of active substances. This opens the door even wider for fraud and misleading data generation. An acceptance of therapies with lacking efficacy and potential risk of aggravating a disease status or harm due to side effects. The cases which generated tremendous harm in the past for many patients and led to more stringent admission procedures seem to be forgotten (e.g. VIOX, Mediator, Lipobay).

4. What changes would you like to see happen to improve access to innovative therapies?

By describing the issues, we have been hinting on some changes already. AIM is of the opinion that a broad political and societal discussion is needed about what a fair price is for pharmaceuticals. Discussions about fairness of prices require transparency of prices, transparency of costs made in the field of R&D (and whether those costs have been made by industry or by the public (public research institutes, universities, through public grants, tax waivers, etc) and require better insight in the efficacy and relative effectiveness of pharmaceuticals. AIM is therefore a strong supporter of international collaboration to strengthen health technology assessment. A fair price also requires a discussion about how the (uncertainty about the) clinical/societal value of a drug could be reflected in the price, together with elements as mentioned before: costs of R&D, return on investment/risk, potential market size for the drug, duration of market exclusivity and ability and willingness to pay.

Early market entry should only be permitted under specific circumstances like life-threatening disease for which no alternative treatment is available. Pay for performance schemes could limit the risk for payers.