EUROPEANS WITH RARE DISEASES NOW GAINING IMPROVED ACCESS TO ORPHAN DRUGS

Leading stakeholders in the effort to combat rare diseases, of which there are as many as 8,000 in Europe, concluded that patients are now beginning to gain improved access to orphan drugs. At the European Health Forum Gastein, a panel of experts reviewed recently established policy measures that the rare disease community has been striving to implement for years, including the development of rare disease national plans. Rare diseases affect up to 30 million people in the EU.

Bad Gastein, 7 October 2011 – A session at the European Health Forum Gastein (EHFG) discussed how health technology assessment methodology is being adapted to evaluate orphan drugs for very rare disease. There are between 6,000 and 8,000 rare diseases, together affecting up to 30 million people in the European Union. Approximately 80 per cent of rare diseases are of genetic origin, and these are often chronic and life-threatening. Over recent years, the rare disease community has strived for these policy changes, which include developing rare disease national plans.

According to Christel Nourissier, Board Member & General Secretary of Eurordis, an NGO alliance of patient organisations and individuals, "all stakeholders in the rare disease community agree that the development of national plans has already improved the situation for patients across Europe. We still face numerous challenges. However, by working together we have our best chance at tackling them and maximising the opportunities that also exist."

Shire Human Genetic Therapies, which sponsored the EHFG-Workshop, was selected from a short-list of companies for a pilot project focusing on the adaptation of orphan drug evaluation with the Advisory Group for National Specialised Services (AGNSS) in the UK. "It is essential that the tools used to make decisions on commissioning are flexible enough to accommodate the evidence base associated with medicines for rare diseases if people living with these diseases are to be enabled access to treatment," Shire's Vice-President & General Manager EMEA, Mark Rothera, said. "At the same time as addressing access barriers, it is important that, as a stakeholder group, we look at the timing of access. More harmonisation across European countries is still needed in terms of early access programmes to ensure that patients are not waiting longer than necessary to be given a means of managing their mostly life-limiting and debilitating conditions."

Prof Dr Günther Leiner, President of the European Health Forum Gastein, has a particular interest in rare diseases. He noted how encouraging it was "to see progress being made, given the tremendous challenge such diseases pose for European health care systems. I believe that collaboration and cross-border networks are the only ways to make a real difference to patients in this area of medicine."

There is widespread support within the European community for action at EU level on rare diseases. A Eurobarometer survey, published this year on World Rare Diseases Day indicated that almost all those surveyed agreed that national health authorities should give support to those suffering from rare diseases (96%) and fully reimburse their medication, even if it is very expensive (93%). Additionally, almost all (96%) agree that resources should be allocated to help people suffering from rare diseases access drugs. The survey was conducted in all 27 Member States between 25 November and 17 December 2010.

The EHFG is the most important conference on health care policy in the EU. This year it attracted more than 600 decision-makers from 45 countries for discussions on the latest developments in health care policy.

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