Position Paper

ORPHAN DRUG SAFETY AND SUPPLY

BACKGROUND

Predicting the demand for an orphan drug is fraught with difficulty; the prevalence figures of patients are very unreliable and ethnically heterogeneous, and the willingness of health providers to reimburse such expensive therapies is often inconsistent.

The IPA appreciates that drug manufacturers must make difficult assessments and undertake complex risk-assessments when forecasting demand for these drugs; a process that precedes the high-cost and long-term investment in production facilities.

But there are times when the risks taken by industry are made in favour of maximising profit and can put the security of drug supply to patients in jeopardy.

No accurate figures for the worldwide Pompe population are available and it's not clear whether current inventories can cope with the demand of a considerable number of newly diagnosed patients or even new markets.

EXPERIENCE

During development of enzyme replacement therapy for Pompe Disease by the Genzyme Corporation, the production process underwent several stages of up-scaling. As the population of treated patients grew at a higher rate than expected, the patients experienced temporary supply shortage resulting in missed or delayed infusions. As a result individual patients and their support groups developed a keen interest in production and supply issues.

IPA POSITION STATEMENTS

Companies should maintain a minimum inventory of 6 months, based on the current number of patients and the anticipated increased demand. New markets should not be sought until the inventory can cope with the anticipated demand. A strategy for expanding markets should include transport and delivery issues and the education of experts to diagnose and treat patients.

Two or more production sites should be established globally, preferably in different countries or continents to avoid any risk of production breakdown by terror attacks or catastrophic natural events, and each should separately source biologic materials to avoid any risk of simultaneous contamination.

Industry should work closely with health providers to ensure that their local inventories are always sufficient and that unforeseen delivery delays do not affect scheduled treatments. In the case of delivery delays, affected patients should be informed as early as possible to avoid unnecessary travel. Patient information should clearly indicate what next actions are to be taken and what is the expected date for the next infusion. The IPA is willing to support communication at all levels.

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