

PRESS RELEASE

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5,000 rare diseases need drugs, but Europe only approves a handful each year

Only seven per cent of drug applications for treating people with rare diseases were approved in Europe between 2000 and 2004, despite the fact that there are currently more than 5,000 conditions needing medication.

Yet during the same period, more than 79 per cent of the other drug applications submitted to the European Agency for the Evaluation of Medicinal Products (EMA) were approved, according to research published in the latest **British Journal of Clinical Pharmacology**.

“It’s difficult to find a balance between the urgent need for drugs for patients with rare diseases and guaranteeing their quality, efficacy, safety and, where necessary, making comparisons with existing drugs” says co-author Professor Silvio Garattini from the Mario Negri Institute for Pharmacological Research in Milan, Italy.

“The lack of reliable methods for evaluating ‘orphan drugs’ in a small number of people probably explains the poor quality of the applications.

“However, it is clear that less stringent criteria are acceptable for orphan drugs than for drugs for more common diseases, particularly in view of the small number of patients.”

Between August 2000, when new legislation came into force, and December 2004, EMA’s Committee on Orphan Medical Products reviewed 255 possible drugs for rare diseases that affect less than five people in 10,000.

Only 18 orphan drugs were approved on the basis of epidemiological data, medical plausibility and potential benefit.

During the same period the EMA received 193 marketing authorisation applications for non orphan drugs and 153 of these were approved.

“However, ten of the 18 orphan drugs approved were authorised under *exceptional circumstances* which means that the dossier was not complete and the Committee required additional studies in order to maintain the marketing authorisation” says Professor Garattini.

More...

Rare diseases covered by the approved drugs included two rare forms of leukaemia, Fabry disease, which affects the body's ability to break down lipids and Wilson's disease, in which copper build-up can damage vital organs.

"In the last 20 years international efforts have been made to encourage companies to develop orphan drugs by providing incentives like tax credits and research aids, simplifying marketing authorisation procedures and extending market exclusivity" adds Dr Jeffrey Aronson, Chair of the journal's editorial board. "Only the last of these incentives is available in Europe.

"The experience in Europe also contrasts sharply with the USA, where there are more incentives - 1,100 drugs and biological products were designated orphan products there between 1983 and 2002 and 231 were approved."

Dr Aronson, Reader in Clinical Pharmacology at Oxford University, also points out that the UK National Institute for Health and Clinical Excellence (NICE) does not generally approve drugs for National Health Service use unless their costs are below £30,000 per Quality Adjusted Life Year. Orphan drugs are likely to cost more than that.

"This study suggests that we need more incentives in Europe to develop orphan drugs and to develop them cost-effectively, so as not to compromise our ability to manage other diseases" he adds.

"The tension between equity and affordability is unbearable and pulls in both directions – those with rare diseases deserve to be treated but those with common diseases should not be expected to subsidise them."

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For further information and press copies of the full paper and editorial, contact
Annette Whibley, Wizard Communications
wordwizard@clara.co.uk

Notes to editors

- Orphan drug development is progressing too slowly. Joppi, Bertele and Garattini, Mario Negri Institute for Pharmacological Research, Milan, Italy. **British Journal of Clinical Pharmacology**. Volume 61.3, pages 355 to 360 (March 2006).
- A Quality Adjusted Life Year is calculated by estimating the total number of life years gained from treatment and weighting each year with a quality of life score to reflect the quality of life in that year.
- The National Institute for Health and Clinical Excellence is part of the National Health Service (NHS). Its role includes promoting better use of resources in the NHS by focusing them on treatments which achieve the greatest health gain.
- The **British Journal of Clinical Pharmacology** is published monthly on behalf of the British Pharmacological Society by Blackwell Publishing. It contains papers and reports on all aspects of drug action in humans: invited review articles, original papers, short communications and correspondence. The Journal, which was first published in 1974, enjoys a wide readership, bridging the gap between the medical profession, clinical research and the pharmaceutical industry. www.blackwellpublishing.com/bjcp