Experts urge UK prime minister to act on 'massive' rises in the prices of drugs for rare diseases

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An open letter from 20 consultants and a patient group published in the British Medical Journal today, calls on the prime minister to take action over a legal loophole that allows drug companies to make easy profits by licensing existing treatments for rare (orphan) diseases.

They argue that the current situation concerning orphan drugs is not in the best interests of patients or the NHS and that the cost to the NHS is likely to be above £10m a year.

The original purpose of this legislation was to encourage drug companies to conduct research into rare diseases and develop new treatments. But, as the rules are currently enacted, many companies simply modify existing drugs and obtain a licence for one or more orphan diseases. This then gives the company sole rights to supply the drugs and to charge what many consider "exorbitant" prices.

One example of the effect of these rules is the drug 3,4-diaminopyridine (3,4-DAP), which doctors have been using for over 20 years to treat two rare muscle diseases at a cost of £800 to £1,000 per patient per year. The company BioMarin has now been issued with a licence to supply the drug (marketed as Firdapse) throughout Europe and charges £40,000 to £70,000 per patient per year - a 50-fold to 70-fold increase. Although Biomarin argues that Firdapse is more stable and reliable than 3,4-DAP, clinicians have not found instability to be a significant problem with

3,4-DAP.

Other examples include hydroxycarbamide (hydroxyurea) licensed for chronic myeloid leukaemia. Using 500 mg capsules, it costs £160 a year to treat a patient with sickle cell disease on an unlicensed basis, but it costs £14,900 a year using 1 g tablets of hydroxycarbamide licensed as an orphan drug for sickle cell disease.

Oral ibuprofen for analgesia costs £0.08 per gram, but intravenous ibuprofen for patent ductus arteriosus (a congenital heart disorder) costs £6,575 per gram, tens or even hundreds of times more than the cost of producing sterile ibuprofen solution for intravenous injection in an NHS facility.

"In the present economic situation it seems vital to ensure that systems are in place to prevent excessive commercial profits being made at the expense of patients and public spending," say the signatories.

They conclude: "Legislation on orphan drugs, far from encouraging the development of new treatments for orphan diseases, is severely limiting the availability of existing treatments. We believe that the Medicines and Healthcare Products Regulatory Agency and Department of Health should not just state the rules but should act now to progress the issue of unfairness upwards, so as to instigate change."

In a BMJ investigation, also published today, Dr Sam Richmond, a consultant neonatologist at Sunderland Royal Infirmary, and a signatory of the open letter, argues: "If drug companies are undertaking research where nobody else was interested – and some are – then a monopoly may be justified. But if it's a product already in use, they should clear off, or sell at a price comparable with the existing price."

Dr Daphne Austin, chair of the UK Commissioning Public Health

Network, says: "It disgusts me, it really does. [Amifampridine] is one of a number of drugs that are not new, but under the legislation have been licensed so that they can be sold for much more money, which is pure profit." She believes the price set for the drug is "indecent" and points out that the extra cost of amifampridine in the UK "is equivalent to that of kidney dialysis for 323 patients."

This view is supported in an editorial, which says that current incentives to licence drugs for rare diseases "are now too generous." Authors Robin Ferner and Dyfrig Hughes believe the NHS "could, and should, make and distribute 'specials' (unlicensed medicines) for rare diseases" and that the GMC "should allow doctors to prescribe a drug that meets the individual patient's needs, but is not licensed for the specific indication, even if a licensed medicine exists for the same indication."

In an accompanying analysis, Timothy Cox and colleagues at the University of Cambridge argue that pricing for orphan drugs hinders access to treatment and may warrant a competition law investigation. They add that current high pricing contravenes the aim of the Orphan Regulation, which states: "Patients suffering from rare conditions should be entitled to the same quality of treatment as other patients."

Provided by British Medical Journal