NICE appraisal of bosutinib (Bosulif)

NICE have published their final recommendation (FAD) on the use of bosutinib for the treatment of adult patients with CML in England. http://www.nice.org.uk/

As expected the NICE Committee has reaffirmed the negative recommendation they made in the draft document (ACD) published earlier this year.

Patients in England will continue to be able to receive treatment with bosutinib via the Cancer Drugs Fund (CDF).

What does CMLSG think of the Appraisal Committee's decision?

As I said in my post in August, I thought the Committee was highly unlikely to alter its draft decision so this confirmation of the draft negative recommendation was expected.

However, we (CMLS group) have managed to achieve some significant concessions which will add to those we have already gained in the past. These might seem unbelievably obvious to those of you familiar with CML and the TKIs used as treatment but, believe me, we have fought for them with great tenacity within the NICE system.

In my opinion, the three key concessions are:

1. That it is possible for patients who are treated with bosutinib following a previous treatment with another TKI; to secure a reduction in their disease load, should they cease treatment, which is lower than that when they started bosutinib treatment.

2. A patient treated with Hydroxycarbamide (sometimes called Hydroxyurea-HU) following a TKI treatment will not survive as long as a patient who has already been treated with a TKI and who is then treated with bosutinib, followed by HU.

3. There is a relationship between a major cytogenetic response (MCyR) and overall survival (OS). In other words if a patient achieves MCyR they are likely to survive CML provided that a response is maintained. MCyR is, in this context, described as acting as a 'surrogate marker' for survival.

More broadly, and to use the Prime Minister's, phrase 'we can't go on like this'.

Two years have now passed since a drug for cancer treatment has emerged from the NICE appraisal process without a manufacturer offering a confidential discount (PAS) to ensure a positive recommendation.

As you can see from the concessions gained we have become ensnared in arguments that make no sense to an informed CML patient, since the answers

are obvious if applied to the overwhelming majority of cases in the 'real world' of treating CML.

What happens next?

Pfizer, the manufacturer of bosutinib (Bosulif), retain the option to appeal the Committee's decision, although any appeals can only be made on a tightly limited set of grounds.

Because of the limited grounds, CMLSg will not to appeal this FAD. Pfizer has until the 15th October to decide if they will appeal. The other option open to them is to submit another confidential discount scheme, a Patient Access Scheme (PAS), for approval by the Department of Health (DH). This follows on from their previous offer of a PAS which the DH rejected.

The timespan for a PAS submission is 16 weeks from the publication of the FAD (2nd October). However, as some of you may be aware, over this time it is likely that significant developments will occur that will affect the funding, price and availability of drugs in the NHS, especially in England.

Factors affecting access to drugs in 2014

The Prime Minster announced a two year successor Fund to the CDF last weekend and this should continue to allow patients in England access to certain cancer drugs, including dasatinib, bosutinib and ponatinib, beyond the current CDF expiry date of 31st March next year.

In January 2014 we will see the introduction of Value Based Pricing (VBP) in England. This means that, in addition to the clinical and cost effectiveness of a drug being subject to an evaluation by NICE, there will be an assessment of the wider societal benefits that a treatment might also offer (like being able to return to work). In addition, there will be an assessment of a drug's possible value to particular patient populations who suffer debilitating, life threatening or life limiting conditions, and where there is a lack of, or there are less effective, treatments available.

The same month will also mark the beginning of a successor Pharmaceutical Price Regulatory Scheme (PPRS). This is an agreement between most of the larger pharmaceutical companies to limit the prices they charge for their drugs. It is based on a 'return on investment formula' for a basket (i.e. more than one) of drugs from their portfolio which the NHS agrees to purchase. Relevant companies who do not join the PPRS are legally required to enter another scheme called the Statutory Scheme.

Currently it is highly uncertain what the actual impact these changes will have, especially when, as they must, they are related to each other.

Pfizer's bosutinib (Bosulif) is caught up in this storm and I doubt we shall discover exactly how it will (eventually) be made available to CML patients in the longer term.

Current availability of bosutinib (Bosulif) across the UK

In the meantime those of us in England must rely on the CDF, whilst those in Scotland must wait for the outcome of the current Scottish Medicines Consortium evaluation in November, for which we made a submission in August. For CML patients in Wales, Individual Funding Requests remain the only means of access to bosutinib.