

NICE 2004/047

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***National Institute for
Clinical Excellence***

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NICE Citizens Council to discuss management of rare diseases and 'ultra-orphan' drugs

The Citizens Council of the National Institute for Clinical Excellence (NICE), which provides public input into the Institute's work, will hold its next meeting in London next week (18 - 20 November 2004).

The 30-strong Council of members of the public will be asked to consider the management of patients with very rare ('ultra-orphan') diseases in the NHS. These rare diseases occur so infrequently that few companies may invest in finding developing drugs to treat the disease or, if they do, the purchase price of the drugs may be high in order to recoup the costs of the original research and subsequent manufacture.

The Council's views will inform the Institute about whether its current methods and processes can be used to assess and appraise the clinical- and cost-effectiveness of drugs for very rare diseases. The Institute has already held a joint conference with the Royal College of Physicians where over 145 delegates looked into the approaches used to assess the clinical effectiveness of orphan drugs and the ethical dilemmas facing the NHS in providing resources for those now appearing on the market. NICE expects to present a report on this topic to the Department of Health in Spring 2005.

Primary Care Trusts, Health Authorities and Hospital Trusts have, in the past, made funding available for some ultra-orphan drugs. These drugs are often expensive and, because these conditions are mainly hereditary, individual PCTs may have a cluster of affected patients in their area. In addition, specialist hospitals may carry a significant caseload because of the expertise of individual members of their staff. The diffusion of these drugs across the country tends to be variable and there is no national policy as to whether and how such treatments should be provided by the NHS.

Professor Sir Michael Rawlins, Chairman of NICE, said: “The Citizens Council has already made a huge contribution by enabling NICE to take the views of the general public into account when undertaking its work. The Council has already looked at clinical need, the issue of age and the confidential enquiries. Now we are asking the Council to consider the issue of orphan drugs. This is a complex issue. People with orphan diseases suffer two misfortunes. They not only suffer from a miserable disease; but it is so rare that either no-one will invest in finding cures or, if they do, they may be very expensive.”

He added, “Citizen’s Council meetings are open to the public which provides an opportunity for the public as a whole to consider this important issue.”

Councillors will hear evidence from, and be able to question, a variety of experts, patients and organisations active in this field. Detailed supporting questions for the meeting are being worked through with the active input of Councillors, and the Council is also contributing to decisions over which witnesses will be called to give evidence.

The meeting of the National Institute for Clinical Excellence's Citizens Council will take place from Thursday, 18th to Saturday 20th November at NICE, MidCity Place, 71 High Holborn, London, WC1V 6NA.

The proceedings are open to the public, though numbers are limited due to space constraints. Members of the public who are interested in attending

should register in advance by calling the event organisers on 0161 200 8000 or emailing bt@visiontwentyone.co.uk

Ends

Notes to Editors

1. For more information, to interview witnesses, Council members or senior staff at NICE please contact Fraser Woodward at NICE on 020 7067 5905 or 07879 846 787.
2. Citizens Council meetings are open to the media and public alike and we will be operating a full press office facility during the course of the meeting.
3. All journalists must register with the press office. Fax and telephone facilities will be available in the press office. **There are space constraints at the venue – to ensure your place we do need to know in advance if you are interested in attending.**
4. The line-up of Council witnesses is being finalised but will be confirmed before the meeting.

About NICE

5. NICE is part of the NHS. It is the independent organisation responsible for providing national guidance on treatments and care for those using the NHS in England and Wales. Its guidance is for healthcare professionals and patients and their carers to help them make decisions about treatment and healthcare. For further information about NICE you can visit <http://www.nice.org.uk>.

About the Citizens Council

6. NICE has established the Citizens Council to find out what the public think about key issues that inform the development of the guidance NICE issues. The thirty Council members reflect the age, social circumstances, ethnic background, regional difference and abilities of a cross-section of England and Wales.
7. The announcement by the National Institute for Clinical Excellence (NICE) in August 2002 that it was seeking to recruit members of the public to sit on the Council resulted in around 4,400 applications. Vision 21, an independent organisation brought in to manage the recruitment process and run the Citizens Council meetings, selected 15 men and 15 women that met all the selection criteria. Drawn from all walks of life that reflect the make up of the population in England and Wales, their ages range from 18 to 76. The names of the thirty men and women who will make up the UK's first Citizens Council were announced on Friday 8 November 2002.
8. For full details of the Citizens Council click on the link below.
<http://www.nice.org.uk/citizenscouncil>