BARTH SYNDROME FAMILY DAY

7th March 2020

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NHS England and NHS Improvement

Overview

- Background to commissioning for rare diseases
- Importance of the patient voice
- Funding of new treatments

Background to commissioning for rare diseases

- There has been a small team responsible for commissioning services and treatments for rare diseases for the past 30 years.
- The range of services commissioned has expanded over time and the commissioning responsibility has moved from the Department of Health (now the Department of health and Social Care) to the NHS.
- Clinical Commissioning Groups commission 80% of NHS services
- Specialised Commissioning, which sits with NHS England commissions for more specialist services and includes commissioning for Rare Diseases
- Highly Specialised Commissioning team and the Rare Disease Advisory Group
- 71 services
- 42 hospitals
- Budget of £500 million 3-4% of total specialised commissioning budget
- Generally services are for fewer than 500 patients and delivered at small number of expert centres
- Small team 4 commissioning managers, manager, medical lead, two admin

Rare Disease Strategy

- The commissioning approach is led by the Rare Disease Strategy
- '*A rare disease is a life-threatening or chronically debilitating disease that affects5 people or fewer in 10,000 and requires special, combined efforts to enable patients to be treated effectively.'*
- *The total number of rare diseases is steadily increasing because genetic research is beginning to explain disease patterns that we did not understand before. Research shows that 1 in 17 people will suffer from a rare disease at some point.1 In the UK, this means more than 3 million people will have a rare disease – so rare diseases are not actually rare.'*

Rare Disease Strategy

- promote equity of access – allowing everyone with a rare disease to follow a clear, well defined care pathway, providing high quality services for every individual through integrated personal care plans
- offer a patient centred, coordinated approach to treatment services, specialist healthcare and social care support which takes into account the needs of patients, their families and others who provide essential support
- deliver evidence-based diagnosis and treatment of rare diseases, developed through the best use of regional and national resources that are easily accessible by patients and professionals
- support specialised clinical centres to provide expert, high quality clinical care and expertise to patients their families and carers and the patient's, multiprofessional health care team
- promote excellence in research and develop our understanding of and treatments for rare diseases
- deliver rapid and effective translations of advances in the understanding of rare diseases into clinical care by creating appropriate infrastructure, care pathways and clinical competences
- deliver effective interventions and support to patients and families quickly, equitably and sustainably
- promote collaborative working between the NHS, research communities, academia and industry wherever possible to facilitate better understanding about rare diseases and how they can be best treated
- support education and training programmes that enable health and social care professionals to better identify rare diseases to help deliver faster diagnosis and access to treatment pathways for patients
- promote the UK as a first choice location, for research into rare diseases as a leader, partner and collaborator

Importance of the patient voice

- Whilst expert clinical teams can offer advice and treatment in many cases, only those living with a rare disease have the direct experience of how it has affected them and their families. We must acknowledge how important the patient's experience and views are to:
  - getting a diagnosis
  - creating a care pathway
  - developing services for people with a rare disease
  - improving our understanding of rare diseases
- Lived Experience is extremely important to commissioning
- Patient engagement is built into every service specification
- Patient groups are invited to the Annual Clinical Meeting
- Services are asked to report on patient feedback

Access to new treatments

- New Treatments coming to the market
- Main pathways
- NICE
  - Technology Assessment (TA)
  - Highly Specialist Technologies (HST)
  - Early Access to Medicines (EAMS)
- What NICE agrees is binding on NHS England
- If NICE thinks it needs more information before it can make a decision
- Effectiveness and cost effectiveness , commercial negotiations are often key
  - Managed Access Agreements, agree to monitor the effectiveness of a treatment over 5 years and then that information goes back to NICE to inform its decision

Access to new treatments

- Sometimes a new indication for a drug is proposed, it will go to NICE if proposed by drug company
- If proposed by clinician as an unlicensed indication it can be considered by NHS England, more common for rare diseases
- NHS England policy process
- Is evidence based
- Limited funding available each year , decisions made in May and November

Despite the funding challenges in the NHS there is money for new effective treatments

Questions