A quick overview of the latest guidance and evidence on current issues such as smoking, sexual health, health inequalities, mental health, obesity, substance abuse, women’s health – in fact anything that you ask for!

If you have a topic suggestion, please email it to Rachel Posaner. Previous searches can be found at: http://bit.ly/1H6QOQk

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**European Commission**

- **Orphan medicinal products** (includes details on the latest drugs receiving orphan designation)
  - Regulation (EC) No 141/2000
  - Flash report - Commission Expert Group on Rare Diseases (12-13 March 2015)

**European Medicines Agency**

- Medicines for rare diseases
- Committee for Orphan Medicinal Products (COMP)
- Orphan designation (guidance and procedures on applying for orphan designation for medicines for rare diseases)
  - Orphan Medicines Figures 2000/2014

**EURODIS (Rare Diseases Europe)**

- About Orphan Drugs (covers access to orphan drugs, patient advocates, drug regulation, the involvement of pharmaceutical companies in the development of orphan drugs, etc.)
- What is an orphan drug? (factsheet)

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**World Health Organization**

- Promoting Access to Medical Technologies and Innovation

**NHS Evidence**

- Market access of orphan drugs and the role of multi-criteria decision making
- Market uptake of orphan drugs - a European analysis
- Orphan drugs, orphan diseases. The first decade of orphan drug legislation in the EU
- Pricing for orphan drugs: will the market bear what society cannot?
- Value-based reimbursement decisions for orphan drugs: a scoping review and decision framework

**HMIC**

- The funding of orphan medicines in the UK.
- Rare diseases and orphan drugs : a comprehensive approach of strategic perspective.
  (Hansen, J: Journal of Communication in Healthcare, vol 5, no 4, Dec 2012, p 199-219.)
- A discrete choice experiment investigating preferences for funding drugs used to treat orphan diseases : an exploratory study.
  (Mentzakis et al: Health Economics, Policy and Law 2011; 6 (3): 405-433 (July 2011))
Department of Health

UK Strategy for Rare Diseases and how it will be implemented

Public Health England

The National Congenital Anomaly and Rare Disease Registration Service (NCARDRS)

Congenital anomaly and rare disease registration service in England: the vision

Office of Health Economics

Orphan Drugs Policies: A Suitable Case for Treatment

Valuing Orphan Medicines Using Multi-Criteria Decision Analysis

Multi-Criteria Decision Analysis to Value Orphan Medicines

Orphanet

About Orphan Drugs

About Rare Diseases

Search tool on orphan drugs (search for specific medicines)

Search tool on rare diseases (search for disease profiles)

Orphan drugs world-wide

Lists of medicinal products for rare diseases in Europe

NHS England

NHS England sets out plans for delivering commitments in UK Strategy for Rare Diseases

Rare Diseases Advisory Group (RDAG)

Rare Diseases Advisory Group (RDAG) papers

U.S. Food and Drug Administration

Developing Products for Rare Diseases & Conditions
2020health

Major new study launched by 2020health aims to shine a light on NHS attitudes to rare diseases.

Patients face postcode lottery in accessing orphan medicines for rare diseases within the UK, warns new report

Orphan Medicines: Special treatment required?

Financing drug discovery for orphan diseases. (Fagnan et al: Drug Discovery Today. 19 (5) (pp 533-538), 2014. Date of Publication: May 2014.)


In the news

Spare a thought for “orphan” drugs: the rare disease medicines that prove health is a numbers game (New Statesman, 2015)

NHS Scotland releases new medicines to treat rare forms of cancer (STV News, 2015)

Science, patients driving rare disease drug research surge (Daily Mail, 2015)

Patients with rare conditions face postcode lottery (Guardian, 2012)

NICE and ‘ultra orphans’ (PMLive, 2014)

Very rare diseases, complex issues: evaluating orphan and ultra-orphan drugs (UK Bioindustry Association, 2014)