



NATURAL, ALTERNATIVE, COMPLEMENTARY AND TRADITIONAL THERAPIES

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EURORDIS.ORG

Did you know?

28,187 plant species recorded as being of medicinal use*

NexoBrid® (from ananas comosus, to treat eschar) authorised by EMA in 2012

Fewer than 16% of herbals with therapeutic effects are listed in scientific journals*

Since 1981, 1,130 new therapeutic agents have been approved for use as medicines, of which 593 are based on compounds from natural sources.

St John' Wort used to induce sleep can reduce the blood levels of immune-suppressants and provoke transplant rejection

State of the World's Plants report 2017, Kew gardens
<https://stateoftheworldsplants.com>

Are we active enough on NATC?

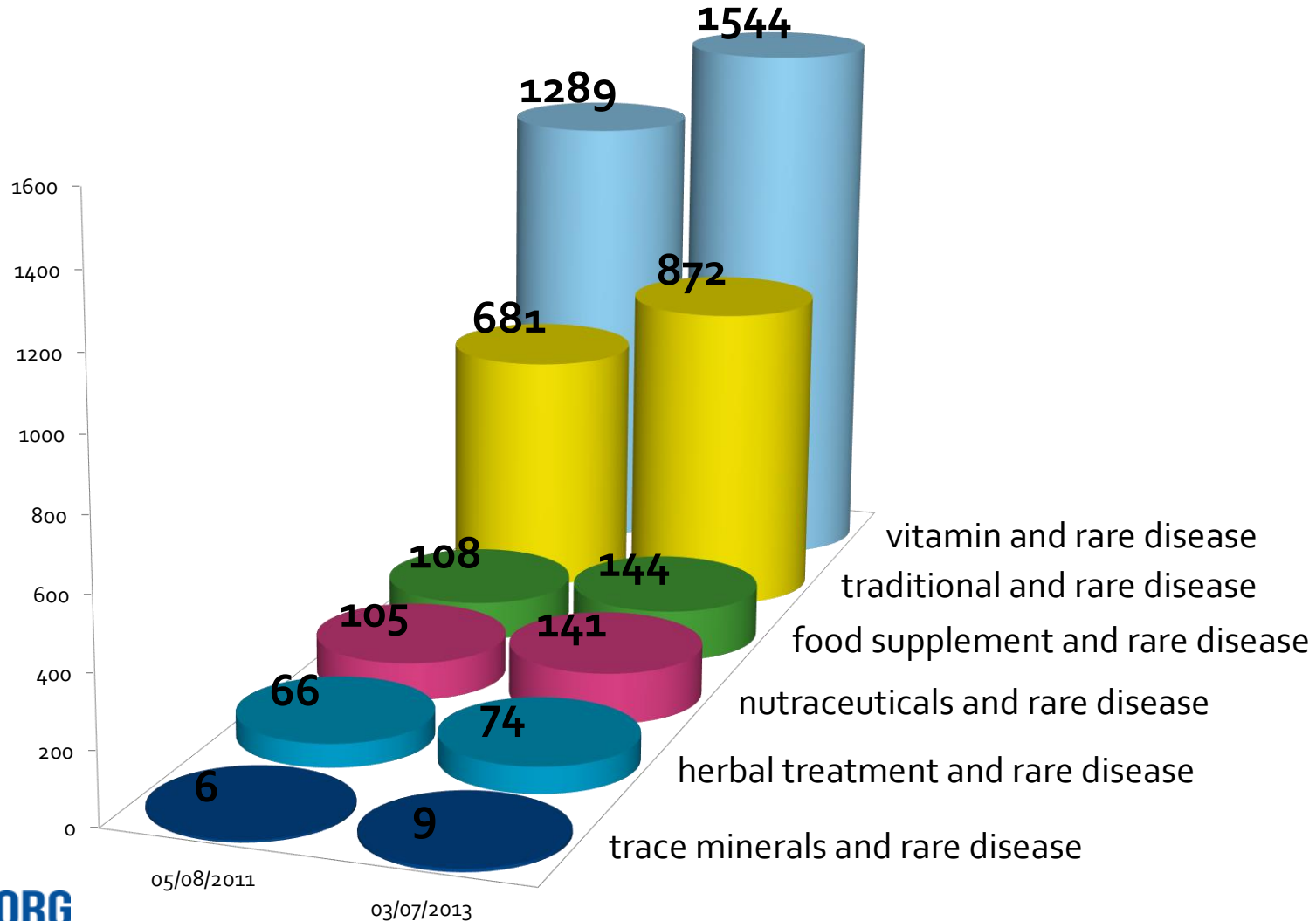


- 2013: DITA task force started a reflection on NATC for rare diseases
- EURORDIS Position Paper: *Health care priorities for 8th FP of EC (2013-2020), Chapter 3.4.: Natural, Alternative, Traditional, Complimentary (NATC) treatments for rare diseases.*
- Attempt to include research on NATC in DG Research priorities
- Development of a research project “TheraBe” / self-disease management
- European Network of Rare Diseases Help Lines meeting 2016, Budapest: a need to identify scientific resources to respond to patients’ questions



Scientific literature review

Scientific papers already published on (PubMed):



The individual therapeutic behaviour of a Retinitis Pigmentosa patient can lead to experiment one or all of the following

- In the 80s: acupuncture, vitamins, ENKAD*, ozone therapy
- Later: “Cuba therapy”, vitamin A palmitate, vasodilators, Chinese herbs, live cell therapy, hyperbaric oxygen therapy
- Today: RetinaComplex, lutein/zeaxanthin, fish oil, valproic acid, curcuma/curry, trans-corneal electrical stimulation of retina (Okuvision)
- Nutrition: fish, spinach, blueberries, red wine
- *Ukrainian preparation of pyrimidine nucleoside 3-phosphate, and oligo-ribonucleosides

None were evaluated

Rainald, DITA task force meeting, Dubrovnik June 2013

Access campaign survey 2016

Questionnaire in 13 languages




INSERM IRB approval July 2015

1,401 valid responses



- Out of 1,291 active substances: 54 were herbal products, food supplements or vitamins
- Representing 4.2% of products reported
- Probably underestimated as respondents were not specifically asked to report such products but “medicines”

Examples

NATC	Condition/indication	Country
 <p>Japanese knotweed (renouée du Japon)</p>	Borreliosis (Lyme disease)	Fra
 <p>Cystinol N (genuine goldenrod, dried extract of bearberry leaves)</p>	Interstitial Cystitis	Fra, Ger
<p>Sodium benzoate</p>	Nonketotik Hyperglycaemia	Fra
 <p>Cornstarch (kukuruzni skrob)</p>	Glycogenosis Type 1 von Gierke	Croatia

And also

- L Carnitine
- Co-enzyme Q
- Calcium
- L-arginine
- Cannabis

Herbal medicines in the African pharmacopeia (Ghana)

Botanical name (Family)	Uses cited	students citing it. (n = 120)
<i>Carica papaya</i> (Caricaceae)	Intestinal worm infestation, malaria, fever, epilepsy, stomach pain, hepatitis, catarrh, typhoid	47 (39.2)
<i>Azadirachta indica</i> (Meliaceae)	Malaria, fever, headache, catarrh	102 (85.0)
<i>Moringa oleifera</i> (Anacardiaceae)	Constipation, headache, immune booster, liver diseases, malaria, hypertension, pains, stomach upset, typhoid	40 (33.3)
<i>Chromolaena odorata</i> (Asteraceae)	Bruises, wounds, stop bleedings, boils	18 (15.0)
<i>Mangifera indica</i> (Anacardiaceae)	Fever, malaria, typhoid, cough	11 (9.2)
<i>Psidium guajava</i> (Myrtaceae)	Malaria, constipation, chicken pox	4 (3.3)
<i>Khaya senegalensis</i> (Meliaceae)	Waist pains, fever, malaria, boils, stomach upset	11 (9.2)

With shortages,
counterfeit,
pharmacovigilance...

This morning

9:30 – 9:40	Introduction	François Houyez, EURORDIS
9:40 – 10:00	Case study: a mushroom to treat muscles?	Tuy Nga Brignol, AFM-Telethon
10:00-10:20	NATC issues in the field of rare retinal disorders	Rainald von Gizycki, Pro Retina Ger.
10:20-11:00	Table discussions	All
11:00-11:30	Coffee break	
11:30 - 12:00	Reports from table discussions (5 minutes)	
12:00-12:20	The role of the HMPC at the EMA	Rob Camp, DITA task force
12:20-12:40	Patient questionnaires, standards and requirements	Sieta de Vries, Univ. Groningen
12:40-13:00	Next steps	All

Next steps

Consequences for life and quality of life

- doubtful expenditure
- everyday stress
- adverse drug effects
- non-compliance with clinical recommendations
- interactions with other medicines
- no individual endpoints
- emotional pressure by family/friends
- uncertainty about impact on disease progression (e.g. worsening of visual field)
- illegal procurement of products

Questions / issues

- Which alternative or complementary treatments do rare disease patients pursue, in particular: acupuncture, antioxidants, nutritional supplements, diets, stem cell experiments, vasodilators...?
- What are the social and medical factors determining the acceptance of new or alternative therapy options, e.g. age, gender, education, stage of the disease, family status, motivation?
- What are the issues and solutions for rare disease patients related to reimbursement of costs for alternative therapies and clinical trials?
- How much out-of-pocket are patients and families willing to pay for not-yet approved alternative or complementary treatments?

To develop a mobile app?

- You're in a shop, you consider buying a product:
 - Consult the literature and other information sources on NATCs
 - Platform for the public presentation of results of clinical studies about NATCs (mHealth) with summaries, or colour codes
 - Information platform: patient friendly presentation of relevant results of clinical studies with NATC for specific rare diseases, and other scientific data
- Report outcomes (PROs)
- Report side effects
- Patient diary with information on non-validated products the patient is using (self-management: electronic patient records and benefit to guide doctor-patient relationship)



Thank you for your attention.

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