
PATIENTS' VIEWS ON THE PRODUCTS SELECTED FOR THE ADAPTIVE PATHWAYS PILOT



EUROPEAN
CANCER
PATIENT
COALITION

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Adaptive pathways workshop, 8 December 2016, London

ECPC: "NOTHING ABOUT US, WITHOUT US"

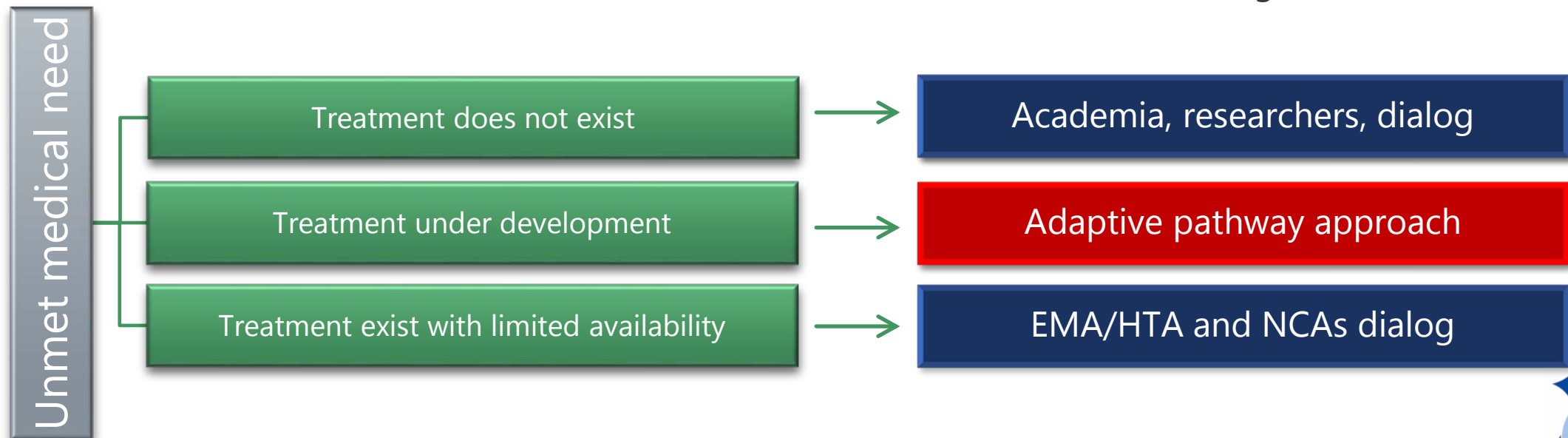
- Representing over 400 cancer patient groups in 46 countries
- All cancers are represented– common and rare cancers
- Run and governed by patients
- Promoting timely access to appropriate prevention, screening, early diagnosis, treatment and care for all cancer patients
- Reducing disparity and inequity across the EU
- Encouraging the advance of cancer research & innovation
- Increasing cancer patients' influence over European health and research policy



THE BASIS OF AP: UNMET MEDICAL NEEDS

„...‘unmet medical needs’ means a condition for which there exists no satisfactory method of diagnosis, prevention or treatment authorised in the Community or, even if such a method exists, in relation to which the medicinal product concerned will be of major therapeutic advantage to those affected”

Commission Regulation (EC) No. 507/2006



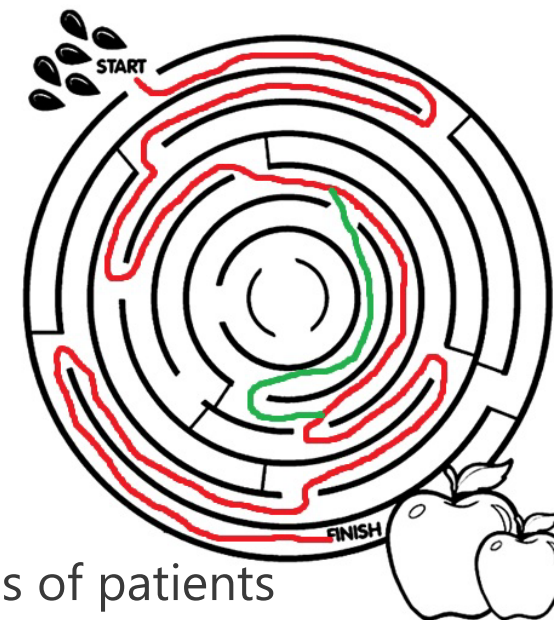
ADAPTIVE PATHWAY

What adaptive pathway is not:

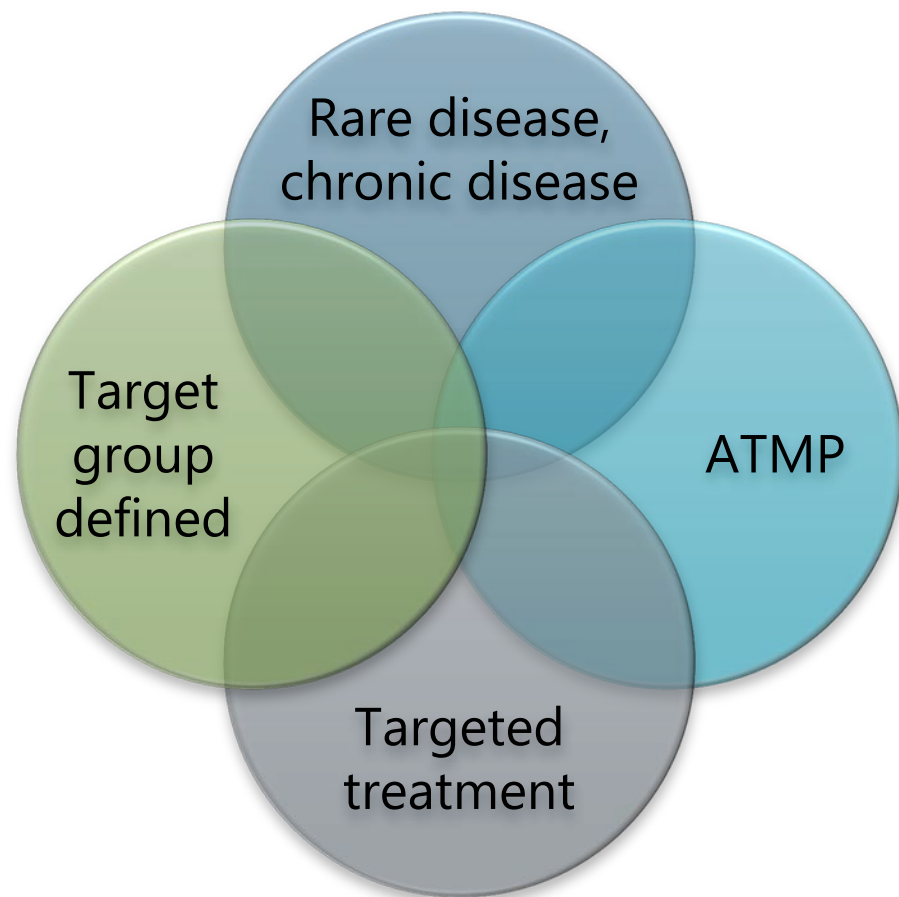
- Standard procedure within marketing authorisation of medicinal products
- „Easier way” or simplification in benefit/risk assessment
- A procedure above all procedures
- A shortcut in medicines evaluation – not a new regulatory tool

What adaptive pathway is:

- An approach to improve timely access to new medicines for targeted groups of patients
- An opportunity to accelerate and facilitate the pathway of product development to potentially achieve earlier access to medicines through an early dialogue involving all stakeholders
- A use of existing regulatory tools to achieve shorter time in approval and/or reimbursement decision in well-defined patients’ populations



PRODUCTS FOR ADAPTIVE PATHWAY



Criteria that identify a good candidate product for AP:

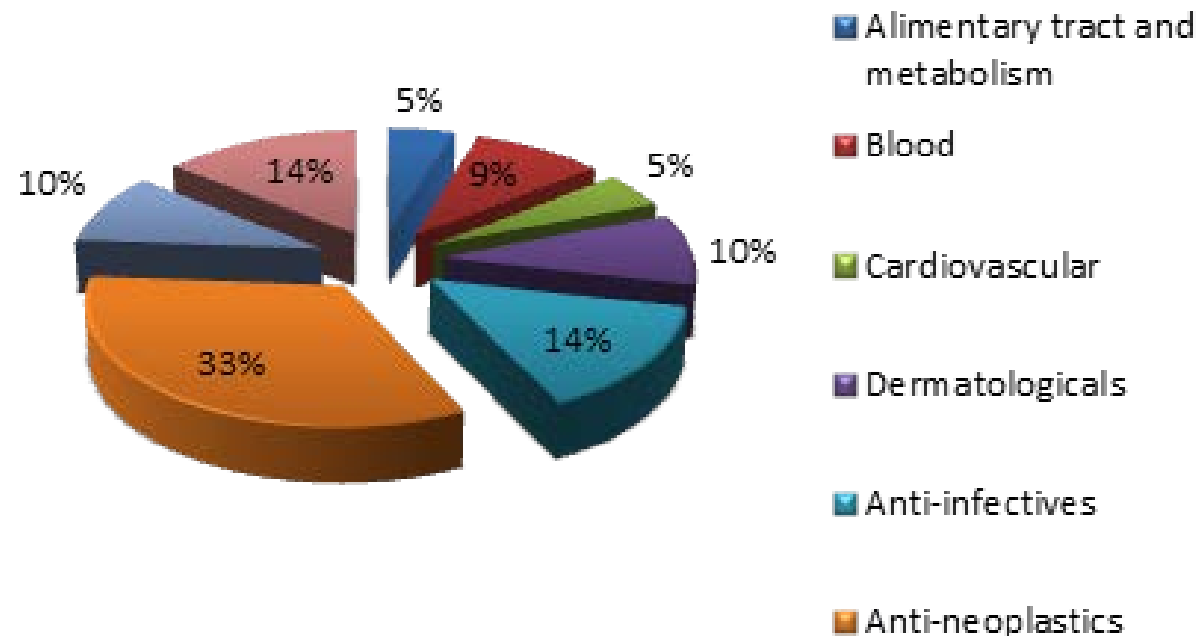
- An iterative development plan (from narrow populations with highest medical needs to wider patient populations)
- Ability to engage HTAs and patients in discussions on a medicine's development
- Proposals for the monitoring, collection and use of real-world data in further medicine's development

AP STATISTICS (RECEIVED FROM EMA)

- 62 products submitted as candidates
- 20 selected for in-depth discussion with company (Stage I) including:
 - 5 orphan medicines
 - 4 ATMP (Advanced Therapy Medicinal Products)
 - 5 anticancer
- 18 proposals selected for Stage II (in-depth meeting after Stage I) including:
 - 6 orphan medicines
 - 3 ATMP (Advanced Therapy Medicinal Products)
 - 4 anticancer

Surprisingly: only 4 SMEs!

Products submitted by ATC code

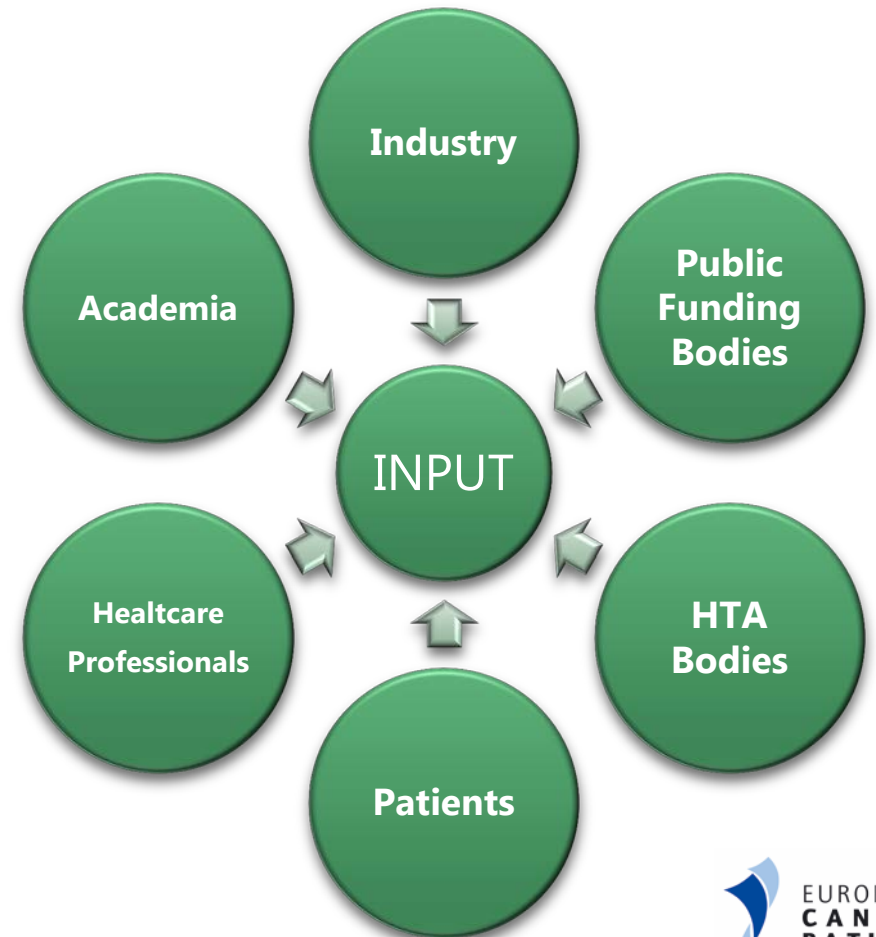


EMA/HTA PARALEL SCIENTIFIC ADVICE WITHIN ADAPTIVE PATHWAY

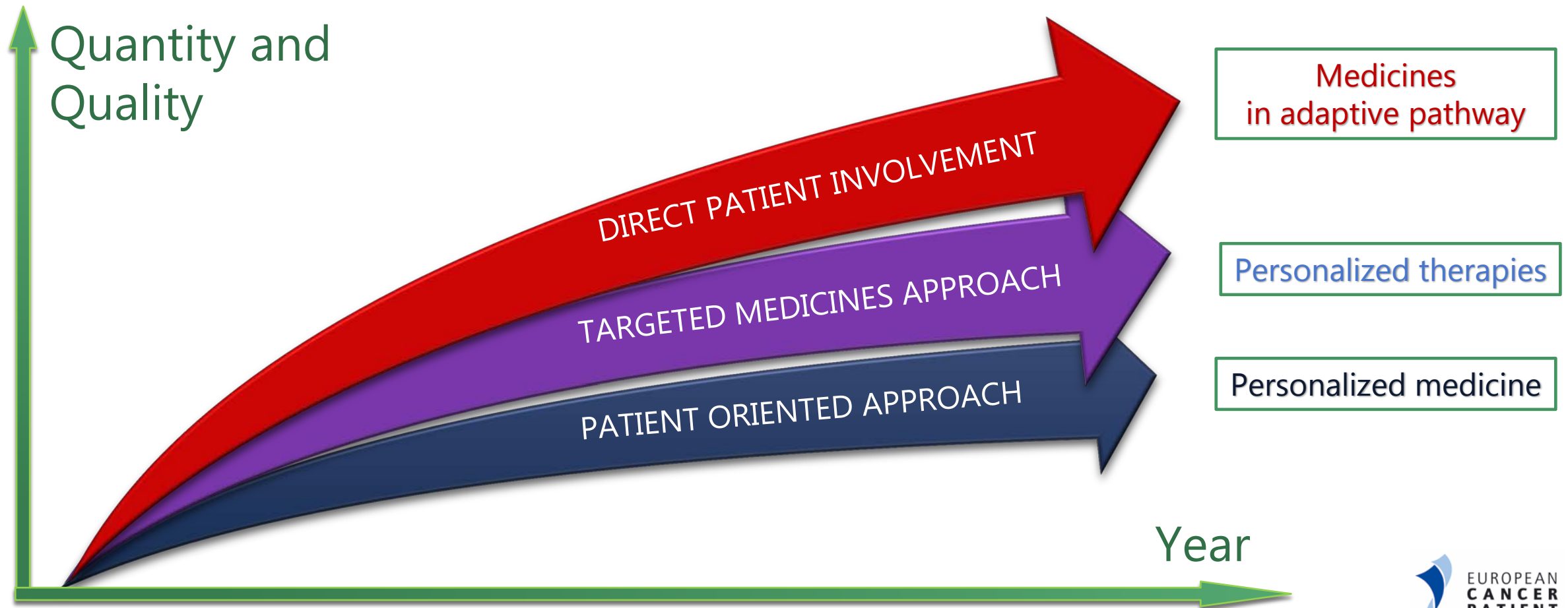
Brainstorming discussion among all relevant stakeholders, including regulators, companies, Health Technology Assessment bodies (HTAs) and patient representatives, to explore ways to optimise the development of requested therapies

Different positions, common dialogue:

- Revision of development plan proposed by the company
- EMA (HPs, researchers): scientific evaluation, ADRs monitoring, real world data use, SACTs vs RCTs
- HTAs: focused on risks, indication, RCTs preferred then SACTs, less on economic evaluation,
- Patient(s): focused on benefits, indication, proper information and interested in follow-up



ADAPTIVE PATHWAY DEVELOPMENT



PATIENT'S RECOMMENDATIONS FOR ADAPTIVE PATHWAY

- Necessary enhancement of resources (human, financial) to develop, facilitate AP accordingly to increasing interest and patients' benefits
- Stronger and sustainable collaboration between regulators (national level) and patients groups – FOLLOW-UP of AP needed!
- Education and improvements on new approach to CTs
- Continuous improvement on existing data use (we live in the world of data, messy data)
- Early involvement of patients in AP selection procedures
- More scientific trust in real world data



ECPC POSITION ON ADAPTIVE PATHWAYS

- ~~ADAPTIVE PATHWAY~~ could be regarded as the only potential lifesaving procedure for patients suffering from rare and ultra-rare cancers
- Current AP scheme needs continuous changes in the traditional approach to clinical trials (randomized and broad clinical trials in large populations of patients) presented by researchers
- Looking for safety above all benefits, instead of getting answers to unmet therapeutic needs - is not a solution!
- In follow-up to AP: patients should have a key voice in the HTA procedures, not only at central level during EMA/HTA parallel scientific advice meetings, but at national levels while AP is considered
- Specific patients' groups (rare/ultra-rare/chronic diseases) force to undertake different methodologies including giving specific information on the treatment options, especially within AP
- *The ultimate decision on medicine use should be left to the patient and her or his doctor/professional!*
- *Let the patient to accept or decline higher risk of treatment!*

ECPC POLICY INITIATIVE: WRITTEN DECLARATION

ECPC'S INITIATIVE OF EMA/HTA PROCEDURES HARMONISATION

- Written Declaration 30/2015: ECPC & 19 MEPs ask European Parliament to take a position on sustainability of healthcare, requesting the Commission to undertake necessary steps to harmonise HTA proces at EU level;
- Amendments to the EMA regulation 726/2004: ECPC supported the amendments to the regulation to pave the way for the EMA to centralise the HTA assessment and increase harmonisation

**APPROVED BY ENVI-
EUROPEAN PARLIAMENT**

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Thank you for your attention

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European Cancer Patient Coalition



ECPCtv



**“You have a rare condition called ‘good health’.
Frankly, I’m not sure how to treat it.”**