The scenario of rare cancers in the EU

Prof Francesco De Lorenzo
ESO-ESMO-RCE Masterclass
25th November 2016
ECPC: Europe’s largest cancer patients’ association

- Representing 408 cancer patient groups in 44 countries
- All cancer types – common and rare
- 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 in cancer research & innovation**
- Increasing **cancer patients' influence** over European health & research policy

"Nothing about us, without us"
European Cancer Patient Coalition’s Activities

**ECPC ACTIVITIES**

- Advocacy
- Capacity Building
- Research
- Partnerships

**ECPC Activities**

- Position papers and policy studies
- Awareness-raising events
- EU institution advocacy

**Research**

- eSMART
- IMI PREFER
- H2020MM04
- IMMUNOSABR
- IMI Web-RADR
- InSup-C

**Partnerships**

- CANCON
- Members on the EC Expert Group on Cancer Control
- EMA’s Patients’ and Consumers’ Working Party
- Joint Action on Rare Cancers
- Pancreatic Cancer Europe
- ESMO/ECCO
- ECIBC/QASDG
- CDDF
- ECC
- UIICC
- EAPM
- OECI

**Capacity Building**

- Working Groups
- ECPC Masterclass
- General Assembly
- Education & Courses
- Advocacy Training

**Advocacy**

- Rare adult solid cancers
ECPC: cancer patients’ recognised voice in Europe

ECPC represents cancer patients within:

- **European Commission**
  - Joint Action on Cancer Control – CanCon
  - **Joint Action on Rare Cancers**
  - **Partner of the EURACAN ERN**
  - European Commission’s Expert Group on Cancer Control

- **European Medicines Agency**
  - Patients’ and Consumers’ Working Party

- **European Parliament**
  - Great collaboration with several MEP champions
  - ECPC has successfully advocated for changes to EU laws
A bit of history: ECPC’s know-how in rare cancers

RARECAREnet project (2012-2015)

- Inception of Joint Action on Rare Cancers
- Crucial work for rare cancer epidemiology and policy
- **RARECAREnet**: Information network of rare cancer patients’ organizations and treatment centres - hub of comprehensive information on rare cancers
  - **145 rare cancer patient organisations** identified and contacted
  - **Online library** on information materials for patients on all rare cancers
  - ECPC organised **tailored workshops** to disseminate the projects results in 4 countries:
    - **Belgium, Bulgaria, Slovenia, Ireland**
How does ECPC keep these organisations engaged?
ECPC’s answer: Working Group on Rare Cancers - WGRC

Why an ECPC internal working group?

- Represent rare cancer patients community in a *democratic, structured and transparent way*
- Ensure the *voice of all rare cancer patients* is heard
- Take up the inheritance of RARECAREnet
ECPC’s Working Group on Rare Cancers - WGRC

- In June 2016, the ECPC General Assembly created an internal Working Group on Rare Cancers (WGRC)
- Call for expression of interest disseminated to all ECPC Members on July 11th 2016 (via newsletter)
- Participation open also to non-members active in rare cancers (RARECAREnet network)
- 35 organisations replied to first call
- First meeting held in October 21st 2016
  - WGRC chaired by Jana Pelouchova, Board Member (CML survivors, Board Member)
  - ECPC offers administrative & policy support to the WGRC
  - Dedicated webpage (under construction) & online platform
  - Funded with own resources
ECPC’s Working Group on Rare Cancers – WGRC

Agreed objectives

• Provide ECPC members & non-members the opportunity to formally & directly contribute and collaborate in the work of the JARC/ERN/RCE.

• Offer a platform to share their knowledge & cooperatively generate expertise to respond to the JARC/ERN needs & requests.

• Give WGRC members the opportunity to use the platform to decide on further topics for discussion.

• Ensure that WGRC members & all ECPC members have a practical & effective instrument to react to the European and national policy challenges related to rare cancers.

• Offer capacity building to national organisations support networking and sharing of knowledge.
The policy panorama for rare cancer patients

- Game-changing initiatives for rare cancers
  - Joint Action on Rare Cancers (JARC)
  - European Reference Networks (ERN)
  - Rare Cancers Europe (RCE)

- Obstacles to access to medicines for rare cancers patients
  - ECPC raised the issue in policy strategy “Challenging the Europe of Disparities in Cancer” (@ ECCO 2015)
  - Successful lobbying the Commission and European Parliament in 2016 to harmonise HTA at EU level (public consultation ongoing)
  - ECPC presented 22 amendments to European Parliament INI Access to Medicines Report (supported by several high-level MEP)
  - “Value of Innovation” paper: new analysis of priorities for cancer patients and existing obstacles (to be launched at ECCO2017 in January)

- Other EU policy files in revision
  - Orphan drugs, off-label, cross border healthcare
Joint Action on Rare Cancers - JARC

- Collaboration between Member States authorities, cancer institutes and patients organisations

- Aim: start from RARECAREnet results to set up model for implementation of the European Reference Network

- Leader: Istituto Italiano Tumori (from RARECAREnet)

- Our role in JARC
  - Horizontal: support to all WPs
  - ECPC has specific tasks, deliverables + budget
  - Connecting JARC to the patient communities
ECPC’s Working Group on Rare Cancers – WGRC
Role within JARC

1. Input on “rare cancers” definition & present issues & experiences of problems in the recognition of rare cancers in their own country
2. Input on challenges related to the quality of rare cancer care experienced in their own country or cross border
3. Ensure that the quality standards for rare cancers promote full patient engagement
4. Input on survivorship & rehabilitation issues for rare cancer patients
5. Define criteria for educational tools & learning programs for patient advocates & communities to reinforce patients & carers’ capacity
6. Disseminate the ECPC newsletter on the JARC.
7. Provide content for the JARC web-site dedicated to patients
8. Disseminate JARC activities in national & international fora
European Reference Networks - ERNs

- New EU initiative included in the Cross Border Healthcare Directive (CBHD)
- Objective: connect centres of excellence (hospitals) to treat patients affected by rare diseases, including rare cancers
  - Make sure that rare disease patients get the best treatment
  - If there are not centres of excellence, “gate point hospitals” will be identified at national level
- Duration: indefinite (forever, hopefully)
- There will be several ERNs. Those involving rare cancer are:
  - EURACAN (10 solid rare cancer types)
  - Haematological disease ERN (including rare blood cancers)
  - Paediatric cancers ERNs
- Commission provides little funding for coordination – Member States pay the rest
European Reference Networks
EURACAN

- The solid tumour rare cancer ERN
- Lead by Lyon Cancer Institute (Prof Blay)
- ~70 hospitals involved
- Covers 10/12 rare cancer families
  - EURACAN is subdivided in 10 sub-ERN per each rare cancer family

- ECPC’s role
  - Link with ePAG
  - Coordination of rare cancer patients’ involvement in ERN
  - Support to local associations involved
Endorsement process is on track in:

- France 7
- Belgium 4
- Czech Rep 2
- Germany 6
- Denmark 1
- Spain 3
- Finland 1
- Hungary 1
- Ireland 1
- Italy 17
- Lithuania 1
- Netherlands 8
- Norway 1
- Poland 1
- Portugal 3
- Sweden 2
- Slovenia 1
- UK 7
Rare Cancers Europe - RCE

- Launched in 2008
- Informal forum, managed by ESMO
- Brings together 32 partners
  - Patient associations
  - Oncologists
  - Academia
  - Industry
- Objective: work on policy/advocacy at EU level
  - 1st and principal product: 39 recommendations on
    - Regulatory barriers
    - Centres of excellence
    - Access to medicines
    - Education
    - Methodological barriers in treating rare cancers
    - Advocacy and information
- RCE is changing its mission: filling the gap between EURACAN and JARC (developments in 2016-2017)
  - ECPC
  - EURORDIS
  - CML Network
  - SPAEN (Sarcoma)
  - IBTA (Brain)
  - Chordoma Foundation
  - GIST Support UK & PAWS-GIST
  - Cancer 52
  - Etc…
Policy issues related to rare cancers
Upcoming law revisions and initiatives

• **Orphan Drug Regulation**
  - Determines the incentives that the EU gives to industry to produce medicines for rare cancers
  - No overall revision, but piece per piece (ongoing – 2017)

• **Paediatric Regulation**
  - Regulates incentives for industry to produce new medicines for paediatric disease (including cancer)
  - In oncology, it did not work (1 drug approved only)
  - The European Parliament is working to push a revision (probable in 2017)

• **Cross Border Healthcare Directive**
  - Establishes the ERNs, facilitates mobility of patients across EU countries
  - Problem: it is not working…
  - Monitoring necessary to take action soon
Policy issues related to rare cancers
Upcoming law revisions and initiatives

- **Access to medicines report**
  - Proposal of the European Parliament to solve the problem of access to medicines
  - ECPC has provided key comments
  - It can develop in a new regulation proposal in 2017

- **Adaptive pathways**
  - Concept: EMA gives fast and simpler approval for innovative medicines for unmet medical needs
  - Different models and pilot projects ongoing: MAPPs, PRIME, Conditional authorisation…
  - Problem: patients not really involved in the definition of unmet needs – results of the pilots are not yet available
  - It could have a crucial impact to make new medicines available
Policy issues related to rare cancers
Upcoming law revisions and initiatives

- Off-label use
  - Concept: a doctor uses a drug out of its original indication (not for the disease it has been approved for)
  - Crucial importance for many rare cancers
  - Problems: scandals and use of off-label to save money. Some decision makers have expressed wish to regulate off-label use
  - Risk: off-label too regulated & lack of access for rare cancer patients
  - Status: discussions ongoing informally within European Parliament / stakeholders, for example www.braincouncil.eu/projects/
### Policy files – Timeframe for action

<table>
<thead>
<tr>
<th>Duration</th>
<th>Stakeholders debate</th>
<th>EU initiatives Future laws</th>
<th>Law revisions</th>
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<tbody>
<tr>
<td><strong>Long term (post 2017)</strong></td>
<td>Off-label</td>
<td>Access to medicines</td>
<td>Orphan Drug Regulation</td>
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<tr>
<td><strong>Medium term (within 2017)</strong></td>
<td></td>
<td>Adaptive pathways</td>
<td>Paediatric Regulation</td>
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<tr>
<td><strong>Short term (next months)</strong></td>
<td></td>
<td>HTA harmonisation</td>
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**Stakeholders debate**
- Off-label

**EU initiatives Future laws**
- Access to medicines
- Adaptive pathways
- HTA harmonisation

**Law revisions**
- Orphan Drug Regulation
- Paediatric Regulation

**Rare adult solid cancers**
A complex scenario

EU laws
- Cross Border Healthcare Directive (CBHD)
  - European Reference Networks (ERN) for rare cancer
    - EURACAN
    - Haematological ERN
    - Paediatric cancer ERN
- Orphan drug regulation
- Paediatric regulation
- Access to medicines
- Adaptive pathways
- Off-label use

Joint Action on Rare Cancers (JARC)

EURORDIS ePAGs

ECPC Working Group on Rare Cancers
A complex scenario
Who does what?

**EURACAN**
- 70+ hospitals
- 10 sub-networks (rare cancer families)
- Objective: treat patients
- Duration: indefinite

**JARC**
- 20+ partners
- Functions like a project (work packages)
- Objective: harmonise method
- Duration: 3 years

**RCE**
- Run by ESMO
- 20+ partners
- Informal collaboration
- Objective: work on policy
- New objective: help connect EURACAN-JARC

**ECPC Working Group on Rare Cancers**
Conclusions

• WGRC answers **ECPC’s need to TRULY involve its rare cancer patients associations in JARC, ERN, RCE**
  • Establishment of the WG approved by the ECPC General Assembly
  • Brings forward the **inheritance of RARECAREnet**

• The position of ECPC on rare cancers is the position agreed within the **WGRC**
  • Democratic
  • **Representative** of different rare cancer types
  • Transparent (specific section on the ECPC website)

• **Facilitates collaboration**
  • ECPC is one-stop-shop for all rare cancer patients organizations, **no matter how small**
  • Open to all organisations willing to join
  • ECPC & EURORDIS have established a collaboration method
Launch of the White Paper
*Value of Innovation in Oncology*

Cancer patients express their opinion on what is meaningful innovation – and how to make it accessible and sustainable

**Amsterdam**
Saturday 28 January
15:00-16:00

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Improving collaboration between Patient Organisations and Cancer Centres

A new, patient-orient method to ensure full collaboration between patients associations and hospitals

**Amsterdam**
Friday 27 January
14:30-16:00

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European Cancer Patient Coalition Annual General Meeting

Europe’s largest gathering of cancer patients

**Brussels**
Friday 16 June – Sunday 18 June

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Nothing about us, without us

www.ecpc.org

info@ecpc.org
Oltre 500 associazioni aderenti

- 165 federate
- 173 sezioni di livello regionale e provinciale
- 48 delegazioni di livello regionale e provinciale
- 85 centri di livello provinciale
- 65 comitati provinciali
- 25 punti informativi
- 9 sezioni estere

- FAVO Lombardia
- FAVO Lazio
- FAVO Puglia
- Comitato Interregionale Piemonte-Liguria-Valle d’Aosta
- FAVO Toscana
- FAVO Campania
- FAVO Sicilia
- FAVO Triveneto

25.000 Volontari
700.000 iscritti a vario titolo
Azione di Lobby: la grande alleanza fra volontariato, società scientifiche, istituzioni
PROSPETTIVE DI CURA E DI RICERCA PER I TUMORI RARI

Palazzo Montecitorio - Sala della Regina
Piazza di Monte Citorio

ROMA, 30 OTTOBRE 2015 - ORE 10.00

Ore 09.30 Registrazione partecipanti
Ore 10.00 Salute Internazionale
Sottosegretario Ministero Salute On. Vito De Filippo
Presidente Commissione Affari Sociali On. Mario Marazziti
Presidente Intergroup Malattie Rare On. Paola Binetti

Ore 10.30 Iniziative parlamentari
Indagine conoscitiva sulle Malattie Rare e risoluzione
On. Paola Binetti

Ore 10.50 La voce dei pazienti: i loro bisogni
Moderatore On. Paola Binetti

I rappresentanti delle associazioni e le loro esperienze
di diagnosi e cura:
Prof. Francesco De Lorenzo
Presidente FAVO
Dr. Angelo Ricci
Presidente FIAGOP
Dr.ssa Fiorella Tosoni
Presidente Associazione Andrea Tudisco

Ore 11.30 Il punto sulla ricerca e sull’assistenza
Moderatore Livio Parisi, giornalista ANSA Salute
Le Malattie Rare nella prospettiva europea: gli ERN
Prof. Bruno Dallapiccola
Ospedale Pediatrico Bambino Gesù, Roma
I Tumori Rari: analogie e specificità rispetto alle Malattie Rare
Prof. Giuseppe Tonini
Policlinico Universitario Campus–Biomedico, Roma

Aggiornamento sui dati relativi ai Tumori Rari in Italia,
nella logica del Registro nazionale
Dr.ssa Annalisa Trama e Dr.ssa Gemma Gatta
Fondazione IRCCS Istituto Nazionale Tumori, Milano
I Tumori Rari in endocrinologia: problematiche cliniche
e innovazione formativa in Facoltà di Medicina
Prof. Andrea Lenzì
Società Italiana di Endocrinologia e Consiglio Universitario Nazionale
L’esperienza delle reti in ematologia
Dr. Marco Vignetti
Fondazione GIMEMA onlus e Policlinico Umberto I
Sapienza Università di Roma
La formazione nel campo dei Tumori Rari: Vera sfida per l’eccellenza
Dr. Paolo Delrio
SIEC, Società Italiana di Chirurgia Oncologica,
Istituto Nazionale Tumori IRCCS “Fondazione G. Pascale”, Napoli
I centri di eccellenza nel campo della chirurgia dei Tumori Rari
Dr. Alessandro Gronchi
Fondazione IRCCS Istituto Nazionale Tumore, Milano
I Tumori Rari: tra reti nazionali e reti europee
Dr. Paolo Casali
Rete Nazionale dei Tumori Rari e Fondazione IRCCS
Istituto Nazionale Tumore, Milano
La necessità di uno specifico riconoscimento per i Tumori Rari
Dr. Giovanni Apolone
Direzione Scientifico Fondazione IRCCS Istituto Nazionale Tumori, Milano

Ore 13.30 Conclusions
La prima mozione, di cui prima firmataria è l'On Binetti, impegna il Governo su molti temi cruciali fra i quali:

- l'inserimento della Rete tumori rari nel SSN, al fine di evitare l'interruzione dell'operatività dell'attuale Rete nazionale malattie rare

- la **formalizzazione di una lista di tumori rari**, sulla base di quella proposta dal gruppo di lavoro del Ministero della salute sulla Rete tumori rari, seguendo le conclusioni del progetto Rarecare

- la definizione di criteri per l'accreditamento di **centri di riferimento** per i tumori rari

- la costituzione di un gruppo di lavoro per l'avanzamento del progetto della Rete tumori rari, coinvolgendo i registri tumori e **le associazioni di volontariato oncologico**

- l'accesso per i malati all’**uso compassionevole dei farmaci** attraverso l'aggiornamento del decreto ministeriale 8 maggio 2003 («Uso terapeutico di medicinale sottoposto a sperimentazione clinica»)

- l'investimento sulla **ricerca clinica**
- la valorizzazione delle eccellenze italiane, per realizzare un monitoraggio efficace degli standard di eccellenza, a livello scientifico, clinico-assistenziale ed organizzativo;
- il supporto alla Commissione europea nella procedura di valutazione e selezione dei centri di riferimento italiani che entreranno a far parte delle European Reference Network su base rigorosamente meritocratica, con indicatori precisi e condivisi;
- la proposta di modelli di integrazione e collaborazione tra i nodi di eccellenza delle reti e i diversi operatori del servizio sanitario nazionale, in modo da favorire la conoscenza reciproca e lo scambio di competenze;
- la verifica che in tutti i tavoli di lavoro in cui si trattano i tumori rari siano presenti i rappresentanti delle associazioni di malati che hanno raggiunto livelli di esperienza e di competenza di riconosciuto valore;
- la facilitazione dell'accesso dei pazienti ai farmaci off-label, utilizzando il cosiddetto fondo Aifa per la ricerca.
Istituzione Organismo di coordinamento e monitoraggio ERN per i tumori rari

Componenti:
✓ DG Programmazione Min Sal
✓ DG Sistema informativo Min Sal
✓ DG Prevenzione
✓ Agenas
✓ INT di Milano
✓ ULSS di Treviso
✓ FAVO
✓ ISS
✓ Rappresentanti tutte le Regioni

Individuati i Centri per i tumori rari solidi ed ematologici in Italia per volume di casi trattati

A breve su:
www.oncoguida.it
Thank you for your attention

e-mail: francesco.delorenzo@ecpc.org

@cancereu

European Cancer Patient Coalition

ECPCtv

Nothing about us without us!