

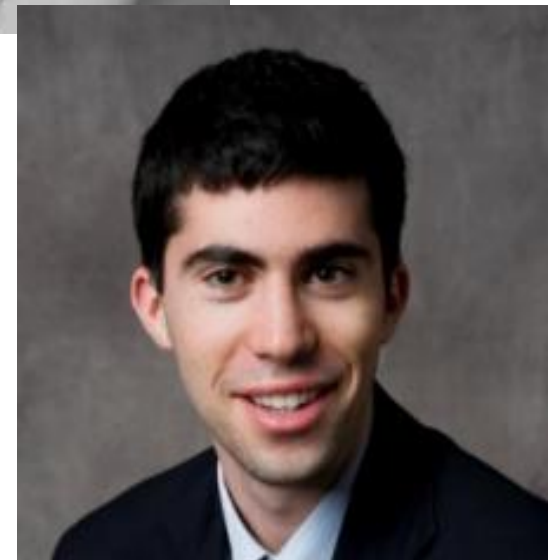
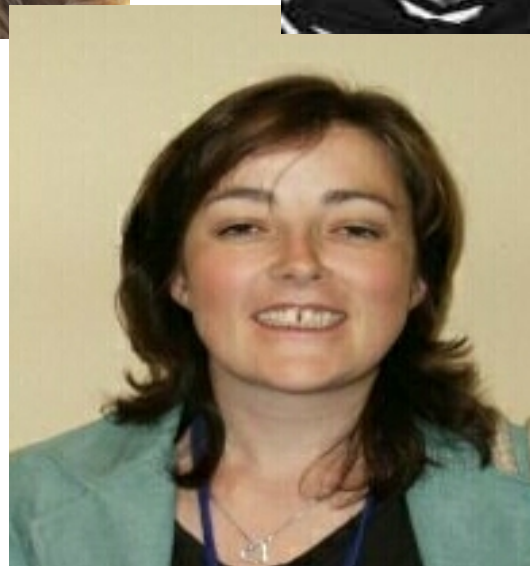


Joining forces for action

Issues facing Rare Cancer Patients in Europe

Jayne Bressington – Patient Advocate

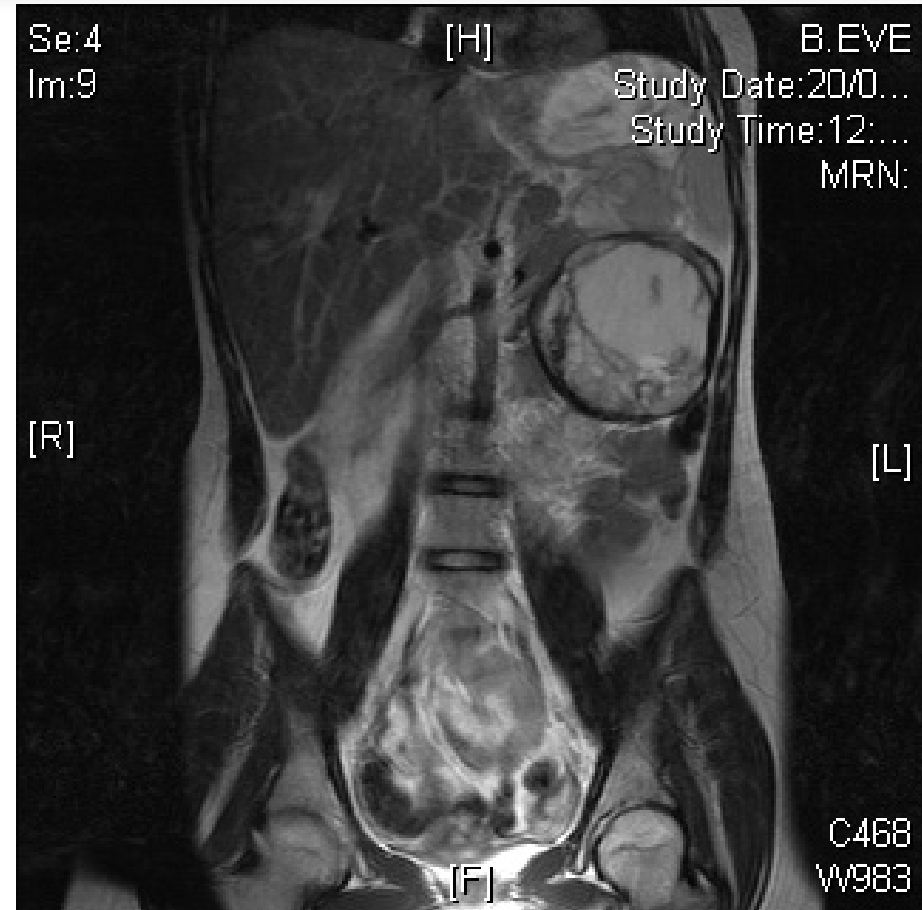
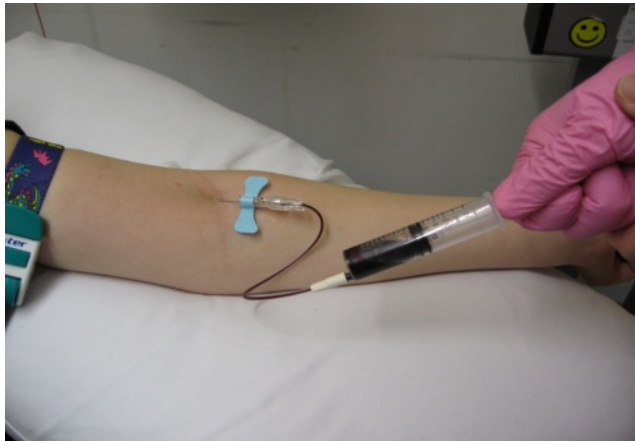
These are Rare Cancer Patients...



“Everyone”
can be a rare cancer patient **“tomorrow”**
One day this was my family.....



Next day we were this.....

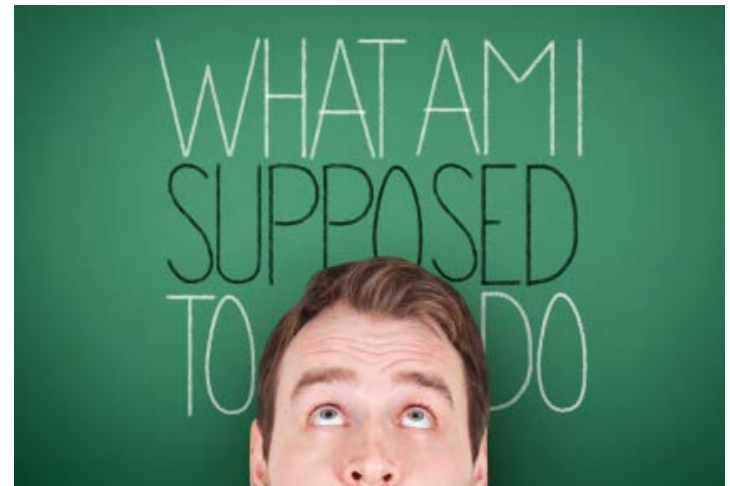


The “**challenges**” that face you when you are diagnosed with a **rare cancer...**

- **Most patients** learn that there is **NO CURE**
- Nobody knows **what causes them**
- They are **not preventable**
- Most doctors have **not heard of them**
- Difficult to find doctors **who are expert in them**
- **Often misdiagnosed / late diagnosis** allowing progression and other “avoidable” disasters
- **Difficult to accrue enough patients** for clinical trials

And when **you try to look further and do something...**

- Little research or funding for research
- You are told that **your numbers are too small to attract help**
- Few treatments and limited access to innovative therapies
- Treatments are **very expensive**
- Difficult to obtain clinical data: Numbers are **small & dispersed**
- Hard to find other patients
- Poor survival rates
- Rarely any **specialist centres with experienced staff**



What is important to rare cancer patients?

- » **Correct diagnosis**
- » **Information and Support**
- » **Effective Treatments and Access to these**
- » **Research to find innovative treatment options**
- » **A cure**

and



- » **Confidence that “the system” is actively geared to support this!**

The most pressing needs...

- » Improved access to existing treatments:
 - **Educate doctors** - recognise rare cancers earlier (primary care / GP's)
 - **Improve diagnosis** – standardise 2nd opinions, scans & mutational analysis

- » An infrastructure supporting translational research / cross border cooperation:
 - **Tumour banks & easy tissue transfer for research**
 - **Clinical trials on an International scale** to maximise patient numbers.
 - **Harmonised European patient data registries**

- » Drug approval by EMA:
 - **New methods and approaches for rare cancers**
 - **Development & protection of Orphan drugs**
 - **Re-use of existing drugs** – already approved for other diseases
 - **A re-examination of benefit v risk**

Continued...

- » Please remove the barriers inhibiting.....
- Cross border transfer of tissue and patient data
- Drugs approval for small groups
- Re-imbursement for off label use of existing drugs across Europe
- Access to patient data – data protection regulation should facilitate access for health research



Help!!!!!! Please remove the **RED TAPE.....**



The patient is the owner of the tissue and data,
so in the end, only his or her consent matters

Rare cancers are.....

» **Fundamental** cancers



- » Understanding **Fundamental** cancers will provide insight and understanding into **Common Cancers**.
- » Rare cancer communities are very good at "**proving the case**" for **innovative research** and can do so with a **very small budget**.
- » **Translational research into rare cancers can serve as a role model.**

What rare cancer patients need in Europe

- » **Cross border collaboration to make small national patient groups become a larger international group.**
- » **Cross border collaboration to offer the best existing treatment to all rare cancer patients**
- » **Remove barriers and use common sense regarding:**
 - **legislation on tissue & bio-banking**
 - **patient data registries**
 - **cross border treatment and re-imburement**
 - **drug approval**
 - **re-use and off label use of approved drugs**
- » **Make it attractive to research into rare cancers in Europe**



Rare Cancers – more common than you think ...



Thank you for your help