



# **Rare Cancers in Europe: Over Four Million Reasons to Initiate Change**

**Presented by  
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Chair and Co-Director, International Brain Tumour Alliance**

**Cancer52  
All Members Meeting  
27<sup>th</sup> January 2015  
House of Lords**



A rare 76 carat diamond valued at  
US \$15 million



Rare kangaroo stamps (Australia)



Rare aurora borealis butterfly  
(South America)



A rare golden snub-nosed monkey (China)

## A landscape of loss...



**M' Liz Keefe and her brother, Joe, three months after Joe's diagnosis of a glioblastoma multiforme brain tumour**



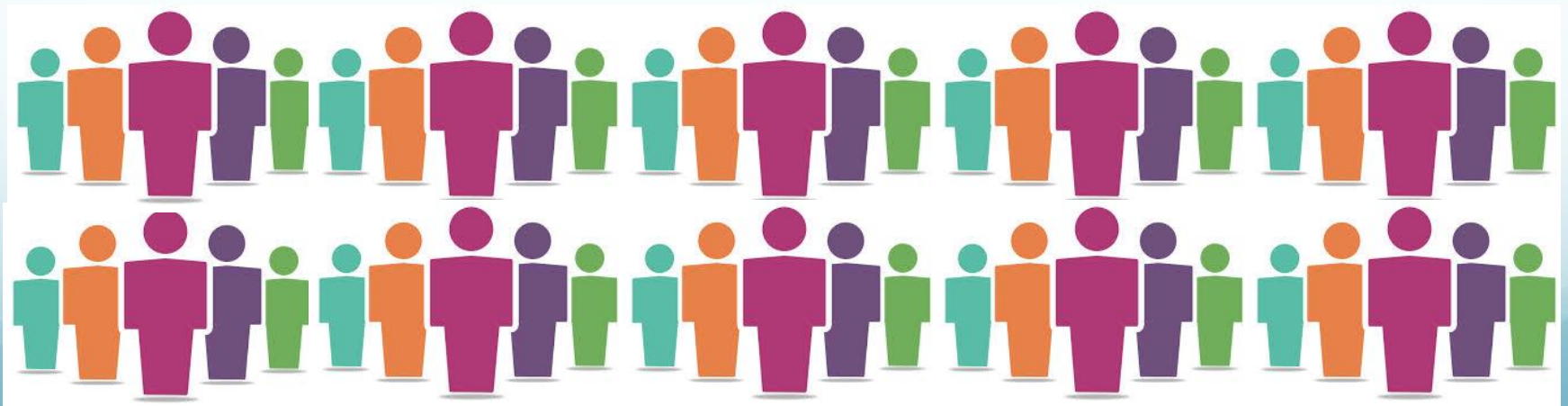
**Painting by M' Liz Keefe**

# Challenges of Rare Cancers

- **Late or incorrect diagnosis**
- **Inequalities of access to appropriate therapies, clinical expertise and care**
- **Shortage of medical expertise**
- **Not enough clinical trials**
- **Lack of interest in developing new therapies due to market limitations**
- **Few available registries and tissue banks**
- **Methodological barriers (in clinical trials)**
- **Regulatory barriers**
- **Lack of information**
- **Lack of support for patients, carers and families**

**Rare cancers = an  
incidence of less  
than 6 people in  
100,000 per year \***

\* Rarecare



# 4.3 million Europeans are affected by a rare cancer\*

\* Rarecare

**This approximately equals the whole population of  
New Zealand (4.5 million) or Croatia (4.2 million)**



**And if every person with a rare cancer in Europe stood next to each other holding hands, then 4.3 million people would stretch 4,000 kilometres which is the entire breadth of Australia, from Perth to Sydney.**

# There are over 180 different types of rare cancers

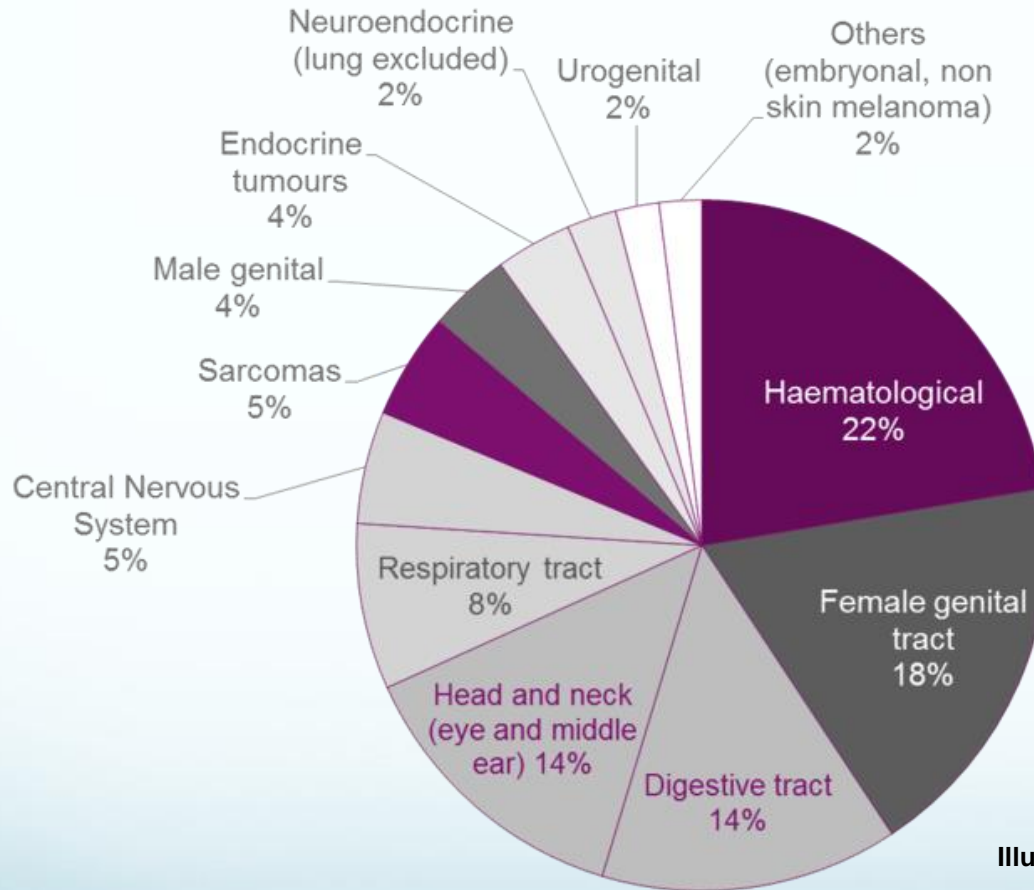


Illustration courtesy of Rare Cancers Europe

## Distribution of “families” of rare cancers

# Rare cancers

Every year in Europe there are about **500,000 new cases** of rare cancer diagnosed.\*

**22% of all cancer cases** diagnosed in the EU each year are rare cancers.\*

Rare cancer (five year relative) **survival is worse at 47%** than common cancer at **65%.**\*\*\*

In the UK, rare and less common cancers account for **54% of all cancer deaths.**\*\*

\* Rare Cancers Europe (RCE)

\*\* Cancer Research UK

\*\*\*Lancet Oncol. Feb:7(2):132-40





## Rarecare:

- Estimated the **burden of rare cancers** in Europe
- Established an operational **definition** of rare cancers
- Created **a list** of rare cancers
- **Statistics** formulated from population-based registries
- Co-funded by the **European Commission**
- Rarecare finished in 2010

## RarecareNet:

- Builds on the experience of Rarecare – provides **updated indicators** of the burden of rare cancers
- Collect and disseminate rare cancer information on:
  1. **healthcare pathways**
  2. identifying qualification **criteria for centres of expertise**
  3. **diagnosis** and **management** of rare cancers
  4. **very rare cancers** so as to provide new knowledge on these diseases
  5. **patient organisations** dedicated to rare cancers

<http://www.rarecaren.net>

Rare Cancers Europe (RCE) is a partnership of cooperating organisations.

RCE works to:

- place the issue of rare cancers firmly on the **European policy agenda**
- to identify and promote **appropriate solutions** to rare cancer challenges
- to exchange **knowledge** and **best practice guidelines**
- foster the creation of **European reference networks (ERNs)**
- to address obstacles to **therapy access**
- to encourage regulatory bodies to include researchers and **patients as equal partners** in the development, approval and assessment of new therapies
- to encourage the use of **alternative statistical methodology** in clinical research
- to establish tools and **frameworks appropriate for supporting a joint patient-physician decision-making process** in conditions of the **high uncertainty** which frequently occurs in the treatment of rare cancers.

[www.rarecancerseurope.org](http://www.rarecancerseurope.org)

# Rare Cancers Europe (RCE) methodological recommendations for clinical studies in rare cancers: a European consensus position paper



The screenshot shows the journal's homepage with the article title prominently displayed. A red banner indicates that the most recent version of the article (mda459) was published on 2015-05-19. The article is listed as 'Accepted Manuscript' and 'Ann Oncol (2014) doi:10.1093/annonc/adv098'. The authors are listed as P. C. Casali<sup>1,2</sup>, P. Bruzzi<sup>3</sup>, J. Bojarski<sup>4</sup> and J.-Y. Blay<sup>4</sup> on behalf of the Rare Cancers Europe (RCE) Consensus Panel. The article was received on July 27, 2014, published on September 18, 2014, and accepted on September 15, 2014. The abstract states: 'While they account for one fifth of new cancer cases, rare cancers are difficult to study. A higher-than-average degree of uncertainty should be accommodated for clinical as well as for population-based decision-making. Rules of rational decision-making in conditions of uncertainty should be rigorously followed and would need widely informative clinical trials. In principle, any piece of new evidence would need to be exploited'.



“We are happy to continue our work with ESMO and RCE to understand exactly where research on rare cancers is struggling so that we can find a way forward.”

Francesco Pignatti, Head of Oncology Evaluation at EMA

<http://annonc.oxfordjournals.org/content/early/2014/09/28/annonc.mdu459.full.pdf+html>

[www.rarecancerseurope.org](http://www.rarecancerseurope.org)



**An international non-profit, non-governmental rare disease patients' organisation representing an estimated 30 million individuals in Europe.**

**EURORDIS** has established a **Policy Action Group (PAG)** on Rare Cancers which is made up of six active volunteers from various rare cancer patients' organisations in Europe.

Some of the EURORDIS PAG members are also members of Cancer52:

- Eric Low (Myeloma UK and Myeloma Patients Euronet)
- Dawn Green (Pseudomyxoma Survivor)
- Kathy Oliver (International Brain Tumour Alliance)

This Policy Action Group will support EURORDIS' European policy work on rare cancers.

**<http://www.eurordis.org>**



# European Commission Expert Group on Cancer Control



**Brings together Europe's top-level decision-makers in the field of cancer, including rare cancers**

(28 EU Member States + Iceland, Norway, Switzerland + candidate countries to EU + EC + industry + academia/cancer prevention + patient representatives)

**Objective:** assist the Commission with

- drawing up legal instruments, policy documents guidelines and recommendations on cancer control
- help prepare guidelines on cancer data including:
  - epidemiology
  - cancer screening
  - quality assurance
  - information on cancer prevention

Eight patients' representatives are on this Commission Expert Group. Two are members of EURORDIS and represent rare cancers:

- Jan Geissler (CML Advocates Network)
- Kathy Oliver (International Brain Tumour Alliance).

**[http://ec.europa.eu/index\\_en.htm](http://ec.europa.eu/index_en.htm)**



CANCER  
RESEARCH  
UK



The International Rare Cancers Initiative aims to “facilitate the development of international clinical trials for patients with rare cancers and encourages the use of innovative methodologies. It also aims to overcome barriers to the organizing of international collaborative trials for rare cancers.”

<http://www.irci.info>



## EMA's Adaptive Pathways Pilot Program

Accelerating patient access to drugs intended to treat serious unmet medical needs.

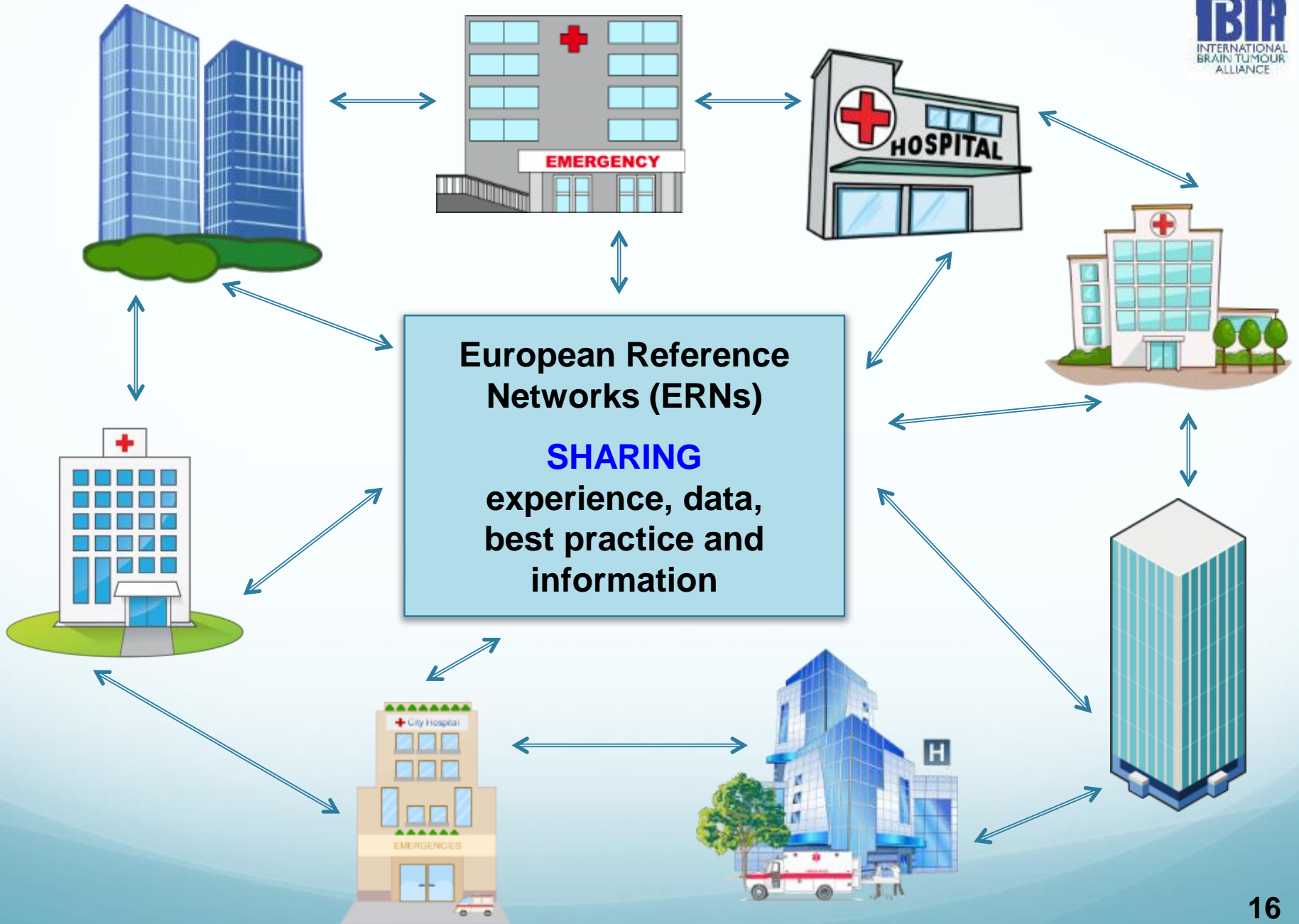
Early approval and licensing of a medicine first for a restricted patient population.

Subsequent progressive adaptations of the marketing authorisation (MA) to expand access to the medicine to broader patient populations based on data gathered in the meantime from its use in the real world and in additional studies.



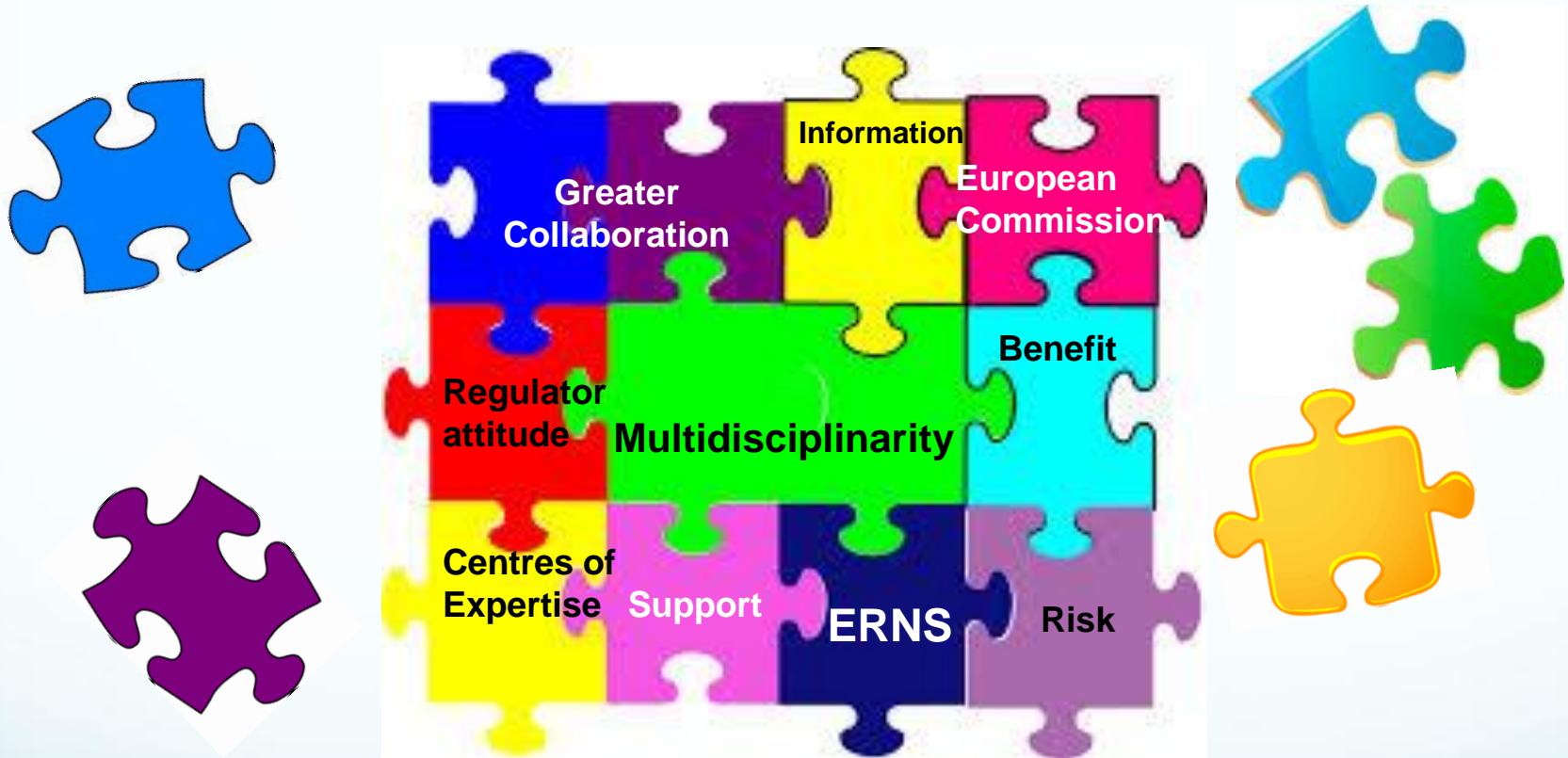
### **SOME CHALLENGES:**

- a greater degree of uncertainty
- greater risk
- buy-in from payers and HTA bodies





# Rare cancers...we're getting there



# Thank you for listening

[www.theibta.org](http://www.theibta.org)

## Learn from the rare!

“Nature is nowhere accustomed more openly to display her secret mysteries than in cases where she shows traces of her workings apart from the beaten path; nor is there any better way to advance the proper practice of medicine than...by the careful investigation of cases of rare forms of disease.”

William Harvey  
16<sup>th</sup> century English physician