



WECAN

Workgroup of European
Cancer Patient Advocacy Networks

Patient access & data collection/ownership: The Patient Perspective

Dr. Rachel Giles (she/her)
President VHL Europa

24 September 2024

Amsterdam, Netherlands



EBA Advocacy Training – Where science meets change





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"Before I write my name on the board, I'll need to know how you're planning to use that data."



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Who we are

**Data
protection**

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&
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**Take home
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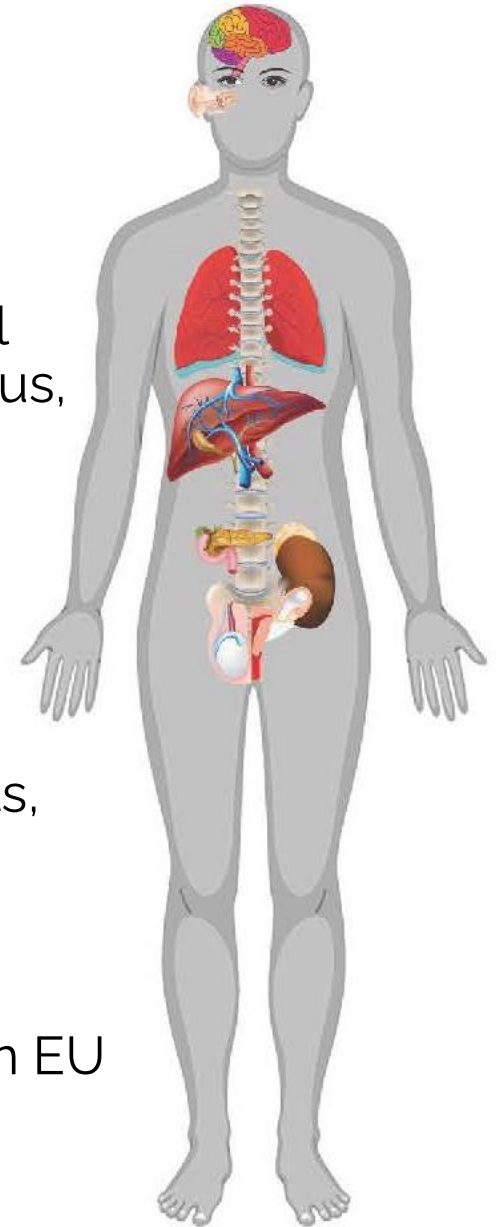


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von Hippel-Lindau

- 1:36,000 people
- Hereditary (20% are first in family)
- Tumors in brain, spine, eyes, ears, adrenal glands, testes, broad ligament of the uterus, pancreas, and kidneys
- Patients need to be constantly scanned, complex care. Starts at birth.
- Specialists: clinical geneticists, endocrinologists, urologists, neuro-oncologists, neurosurgeons, nephrologists, pancreatic surgeons, andrologists, ophthalmologists, etc.
- In the Netherlands we have 460 patients; >300 belong to our patient organisation. In EU 27, 12.5K patients.





- Referral
- Triage
- Registration
- Diagnosis
- Additional Tests
- Treatment
- Follow-up

Care pathway developed

Cancer

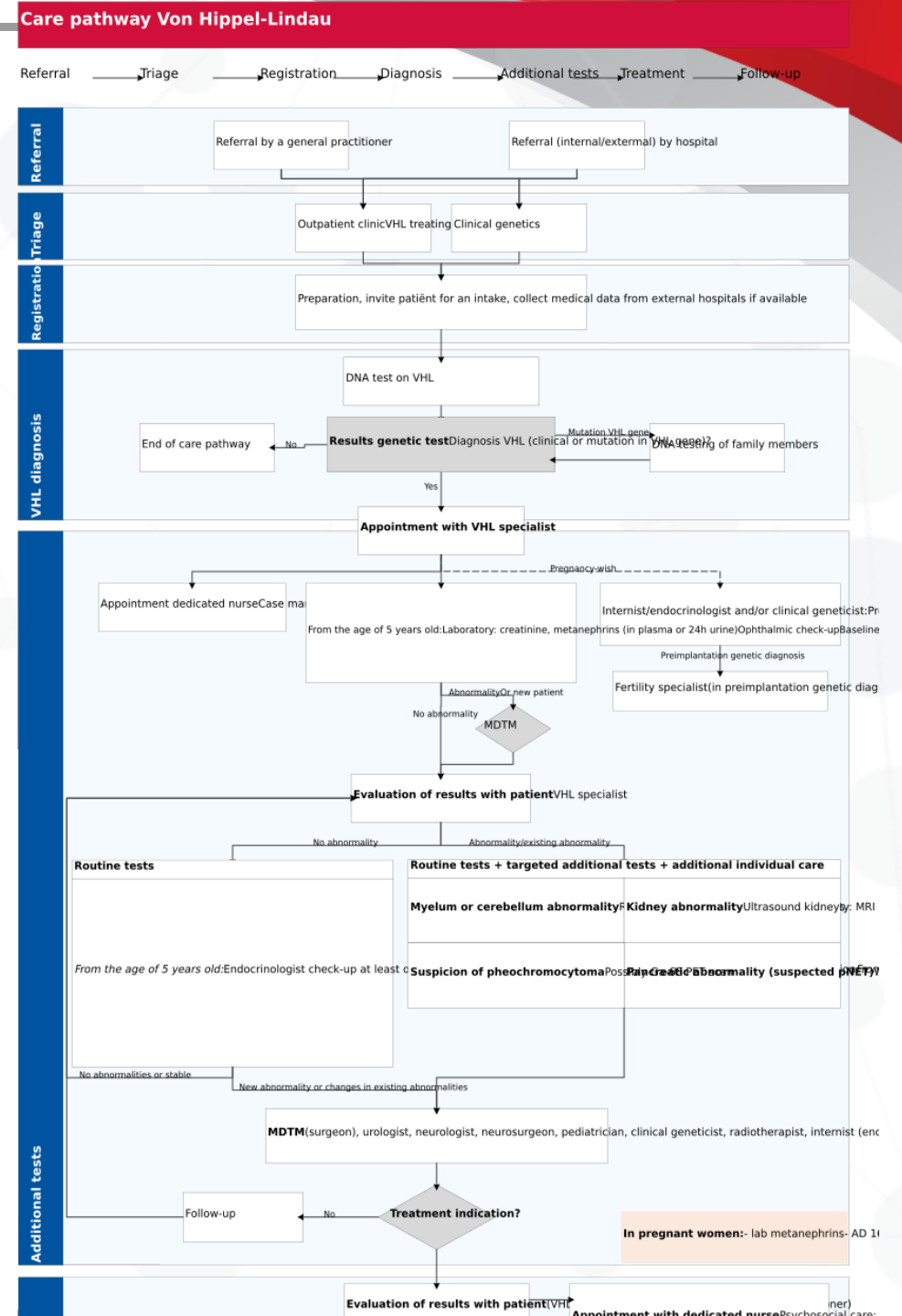
An International Interdisciplinary
Journal of the American Cancer Society

Consensus Statement | Open Access |

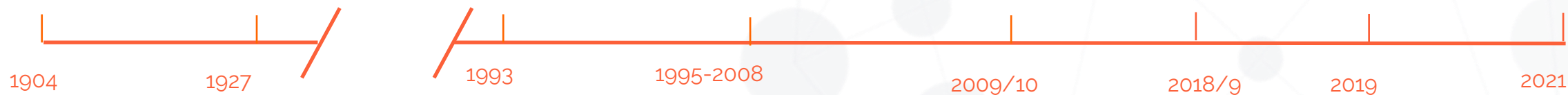
Multidisciplinary integrated care pathway for von Hippel–Lindau disease

Wendy P. G. Wolters MSc, Koen M. A. Dreijerink MD, PhD, Rachel H. Giles MD, PhD,
Anouk N. A. van der Horst-Schrivers MD, PhD, Bernadette van Nesselrooij MD, MSc ... See all authors

First published: 17 May 2022 | <https://doi.org/10.1002/cncr.34265> | Citations: 4



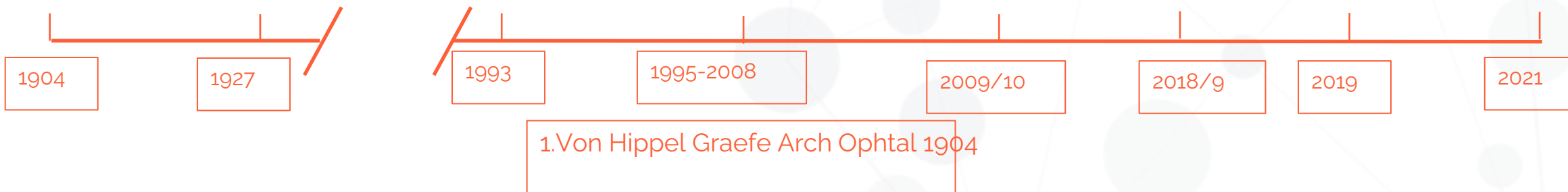
VHL Disease - A Century of Discovery



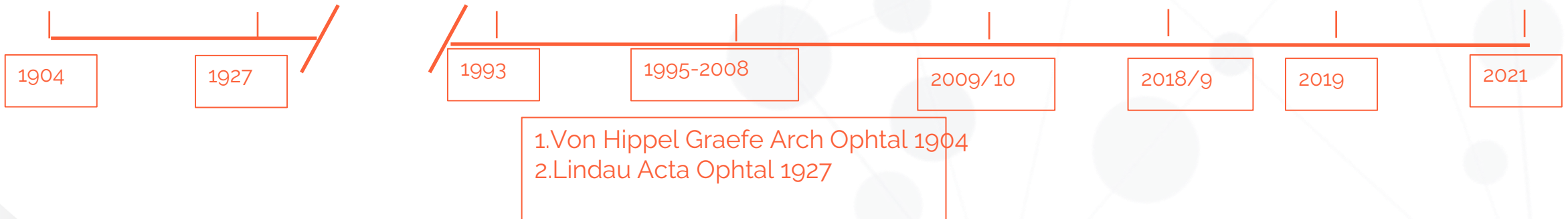
VHL Disease - A Century of Discovery



Eugen
von Hippel

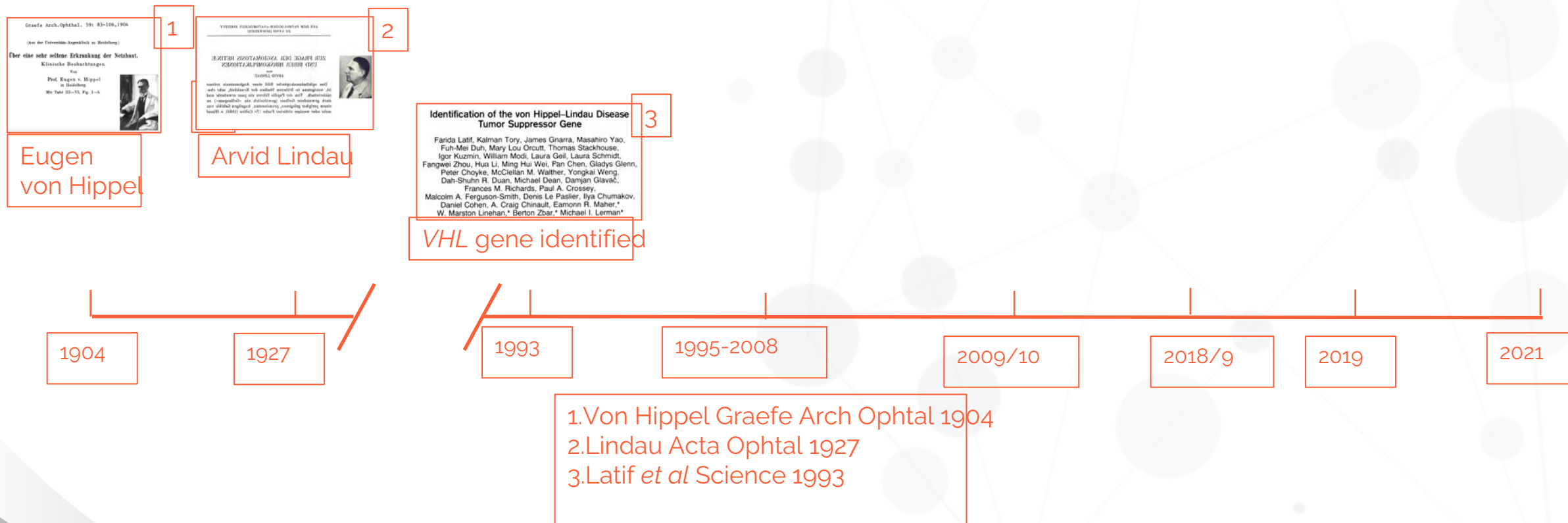


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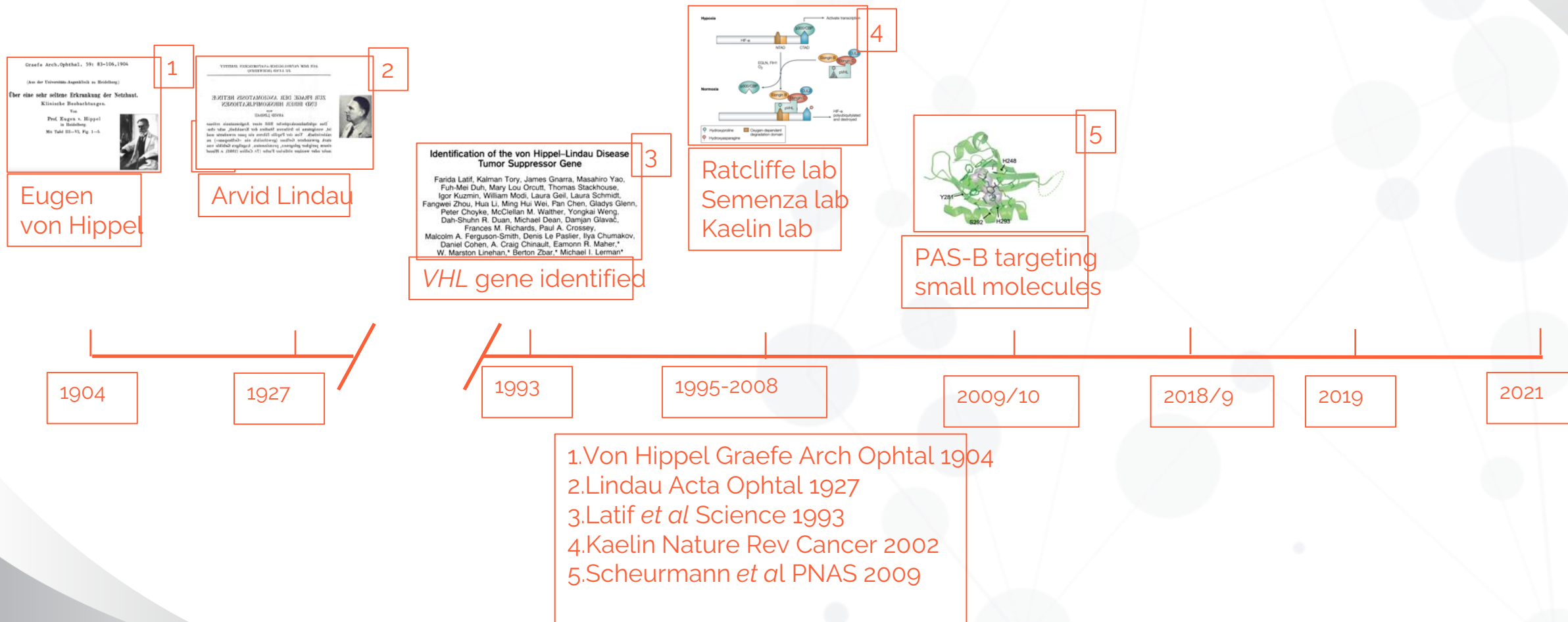
VHL Disease - A Century of Discovery

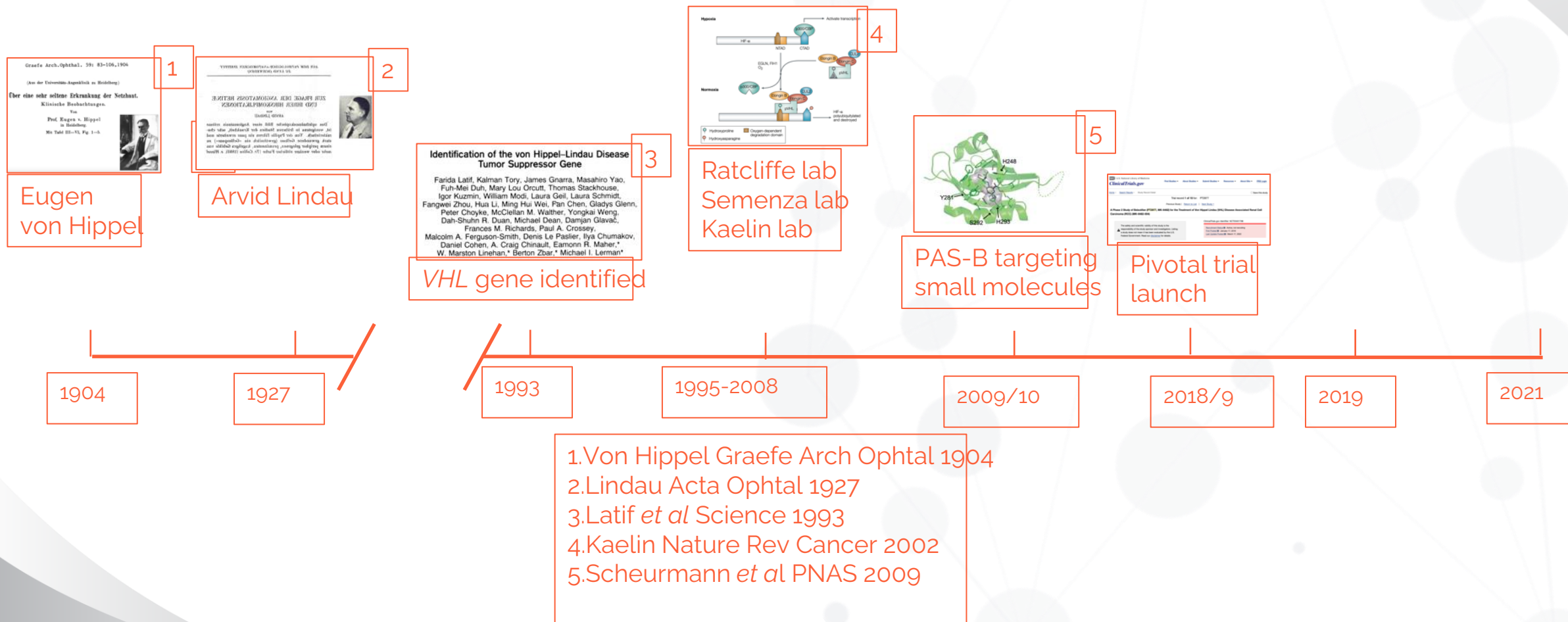
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VHL Disease - A Century of Discovery

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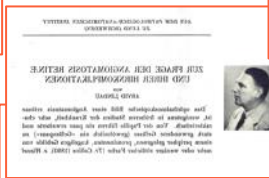




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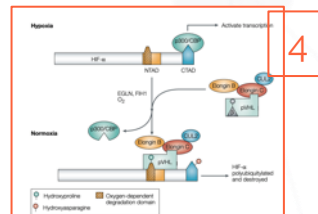
Eugen von Hippel



Arvid Lindau

Identification of the von Hippel-Lindau Disease Tumor Suppressor Gene
Farida Latif, Kalman Tory, James Gnarr, Masahiro Yao, Fuh-Mei Duh, Mary Lou Orcutt, Thomas Stackhouse, Igor Kuzmin, William Modi, Laura Geil, Laura Schmidt, Fangwei Zhou, Hua Li, Ming Hui Wei, Pan Chen, Gladys Glenn, Peter Choyke, McClellan M. Walther, Yongkai Weng, Dah-Shuhn R. Duan, Michael Dean, Damjan Glavac, Frances M. Richards, Paul A. Crossey, Malcolm A. Ferguson-Smith, Denis Le Paslier, Ilya Chumakov, Daniel Cohen, A. Craig Chinault, Eamonn R. Maher*, W. Marston Linehan*, Berton Zbar*, Michael I. Lerman*

VHL gene identified



Ratcliffe lab
Semenza lab
Kaelin lab



PAS-B targeting
small molecules



Pivotal trial
launch



Peter Ratcliffe



William Kaelin



Gregg Semenza

1904

1927

1993

1995-2008

2009/10

2018/9

2019

2021

1. Von Hippel Graef Arch Ophtal 1904
2. Lindau Acta Ophtal 1927
3. Latif *et al* Science 1993
4. Kaelin Nature Rev Cancer 2002
5. Scheurmann *et al* PNAS 2009

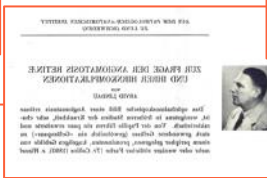


VHL Disease – EU since 2021

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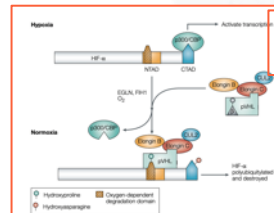
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VHL gene identified



Ratcliffe lab
Semenza lab
Kaelin lab



PAS-B targeting small molecules



Pivotal trial launch



Peter Ratcliffe



William Kaelin



Gregg Semenza



FDA approval



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4. Kaelin Nature Rev Cancer 2002
5. Scheurmann *et al* PNAS 2009
6. Jonasch *et al* NEJM 2021





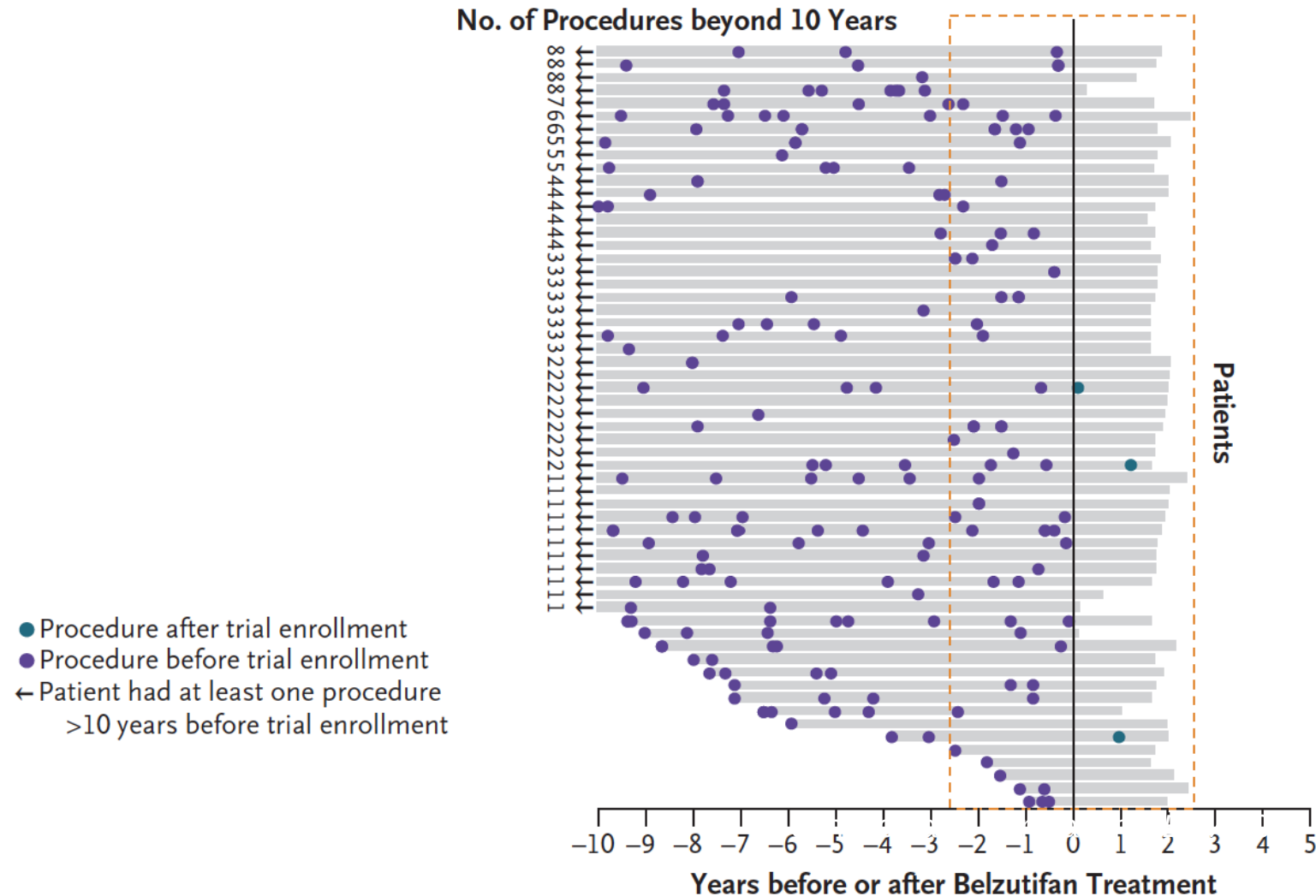
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Tumor reduction procedures before and after treatment initiation for individual patients

Jonasch et al, NEJM, 2021

D Tumor-Reduction Procedures



No. of Procedures per Respective Year 142 18 7 28 15 19 13 15 18 28 24 2 1



EBA Advocacy Training

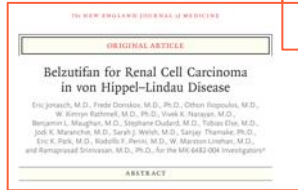


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VHL Disease – since 2021 (EU)

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FDA approval

My personal experience:
12 Patients have died
3 Patients have become Type 1 diabetics
3 Patients have joined trials
90% of questions are about access
>1000 Patients have flocked to support groups
100% Patients are frustrated!
Role has changed from navigator to
expectation manager





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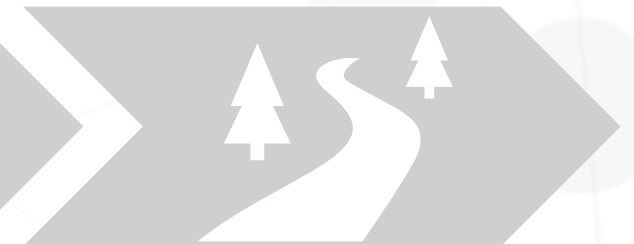
Who we are



**Data
protection**



Research
data collection
&
data analysis



Take home
messages



Who plays a role under the GDPR?

The data controller

Natural or legal person (e.g. patient organization, hospital, company), public authority...



Determines (alone or jointly with others) the **purpose (WHY) and means (HOW)** of the processing of the personal data

The data processor

Natural or legal person (e.g. IT company)



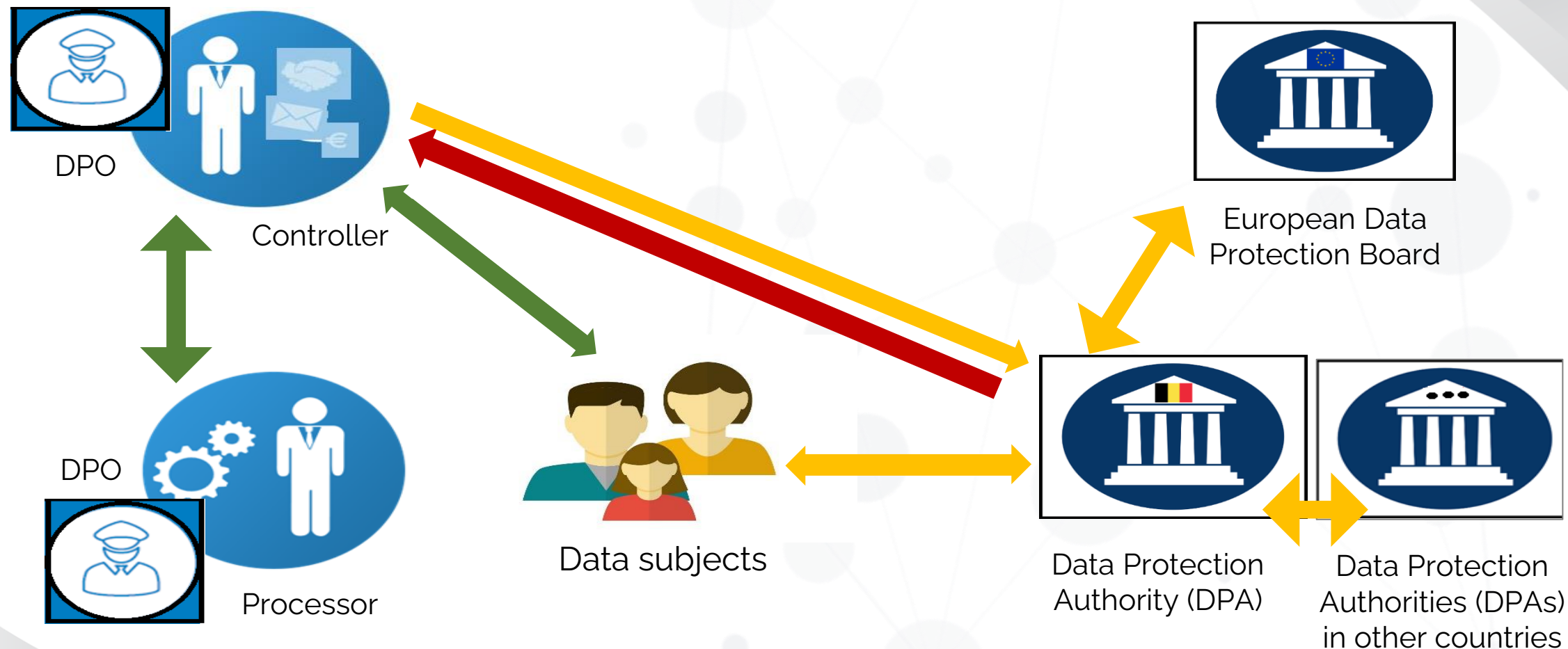
Processes personal data **on behalf of the controller**



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What is the interaction?





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Obligations:

- Controller is responsible and must be able to **demonstrate compliance** with the GDPR principles
- **Data protection by design & by default:** both in the planning and in the implementation phase, data protection principles and appropriate safeguards must be addressed and implemented
- Keep a **record of processing activities**
- **Cooperate with the DPAs**
- **Data security:** implement appropriate technical and organisational measures to protect personal data against accidental or unlawful destruction (e.g. encryption, pseudonymization)
- **Reporting data breaches** to DPAs, **notifying the data breaches** to data subjects
- **Appoint a DPO**
-



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Example 1



About VHL

MyVHL

MyVHL: Patient Natural History Study

Share your story and help find a cure for VHL

Help researchers unravel the mysteries of VHL while learning more about how your VHL journey compares with others. Your information and input is needed to help researchers improve the quality of life for patients living with VHL and ultimately, lead to a cure.

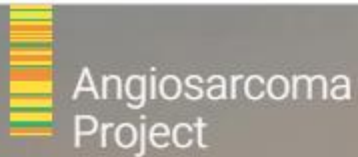
[Register Now](#)

MyVHL is available in English and Spanish.

- >1000 pts
- About to be refurbished
- Pts submit scans and info, or allow hospitals to submit records
- Researchers request access with query



Example 2



Enrollment is now CLOSED for the
Angiosarcoma Project
(ASCproject).

[nature](#) > [nature medicine](#) > [letters](#) > article

Letter | Published: 10 February 2020

The Angiosarcoma Project: enabling genomic and clinical discoveries in a rare cancer through patient-partnered research

[Corrie A. Painter](#), [Esha Jain](#), [Brett N. Tomson](#), [Michael Dunphy](#), [Rachel E. Stoddard](#), [Beena S. Thomas](#), [Alyssa L. Damon](#), [Shahrayz Shah](#), [Dewey Kim](#), [Jorge Gómez Tejeda Zañudo](#), [Jason L. Hornick](#), [Yen-Lin Chen](#), [Priscilla Merriam](#), [Chandrajit P. Raut](#), [George D. Demetri](#), [Brian A. Van Tine](#), [Eric S. Lander](#), [Todd R. Golub](#) & [Nikhil Wagle](#) 

[Nature Medicine](#) **26**, 181–187 (2020) | [Cite this article](#)



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Example 3: Biobanks established by patient groups

- **Association Française contre les Myopathies (AFM)**
 - Neuromuscular diseases. Founded DNA biobank in the mid-1990s
- **Patient Tumor Bank of Hope (Germany)**
 - PATH founded by breast cancer survivors, intended for the sole purpose of supporting breast cancer research
- Patient group retains decision-making power over the biobank, whilst maintaining a tight partnership with the scientists involved

<http://www.genethon.fr/en/rd-2/dna-and-cell-bank>

<https://www.path-biobank.org/>





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Example 3 cont'd: Patients as part of Governance of a biobank

Wales Cancer Bank, UK (publicly funded)

- A Steering Group established the biobank
 - 4 lay members on steering group, established patient & ethics committee
 - Later reimagined to 'Lay Liaison & ethics group'
- Reviews proposals:
 - electronic consenting, volunteer consenting
- Role in Access procedures:
 - researchers do not receive samples until the reviewing lay member is satisfied that content & structure of lay summary describes the proposed research in terminology suitable for a lay audience

Mayo Clinic Biobank (USA)

- Uses deliberative democracy techniques to involve patients and research participants in the policy and decision-making of the biobank
 - Community Advisory Board





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Data
protection



Ethics



Research
data collection
&
data analysis



**Take home
messages**





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Considerations for Patient-owned data

- **Patients/Patient Organisations have the capacity to collect and own data**
- **We need to engage with experts in qualitative and quantitative** research and analysis (key role: **statisticians**). There is loads of room for collaboration!
- We do our best to Implement rigorous **technical and organisational measures** to protect, store and organise data.
- **We use the evidence:** identify 1) issues to focus on, 2) potential solutions, 3) unanswered questions...





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Thank you for your attention!



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