Selecting the treatment – my patient and statistician perspectives

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Patient advocate and statistician
EFSPI regulatory workshop
12 September 2024
Basel

Disclaimer

- All views are my own and they don’t necessary present the views of
  - any of my employers
  - any patient advocacy groups
  - any other patient
- I’m presenting things as I perceived them as a patient using my statistical knowledge :
  - I made mistakes in both roles
  - I made learnings in both roles

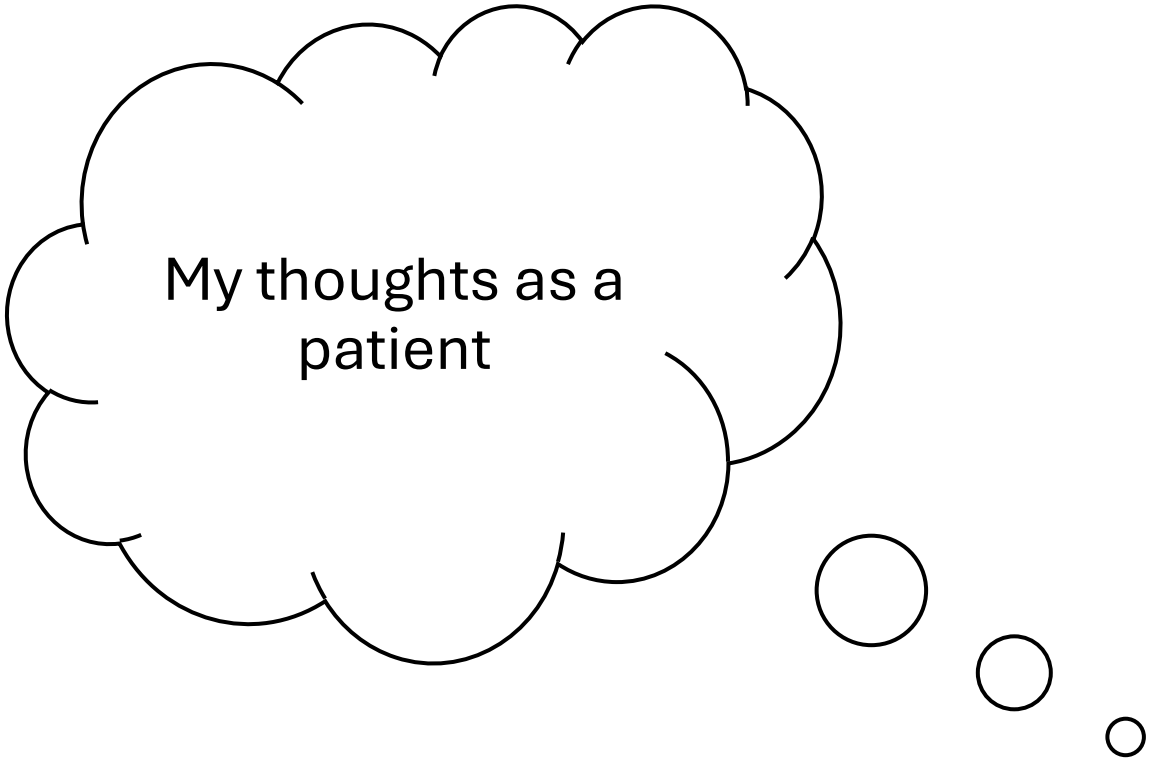
Acknowledgement

- Lucy Rowell
- Tiina Kirsilä
- Jenny Devenport
- Julie Jones
- Florian Posch
- Elina Asikanius
- Heini Alsio

About the speaker

- Current position: Associate Director, Biostatistics BMS
- 2009-2022: Various roles at StatFinn and Novartis
- Education:
  - MSc 2009, University of Turku, Finland
  - PhD 2022, University of Hohenheim, Germany

The mind of a patient



Content

1. Patient journey and prognosis
2. As a patient at work
3. Relevant data – my patient and statistician perpectives
4. Summary

Beginning of the patient journey 2017

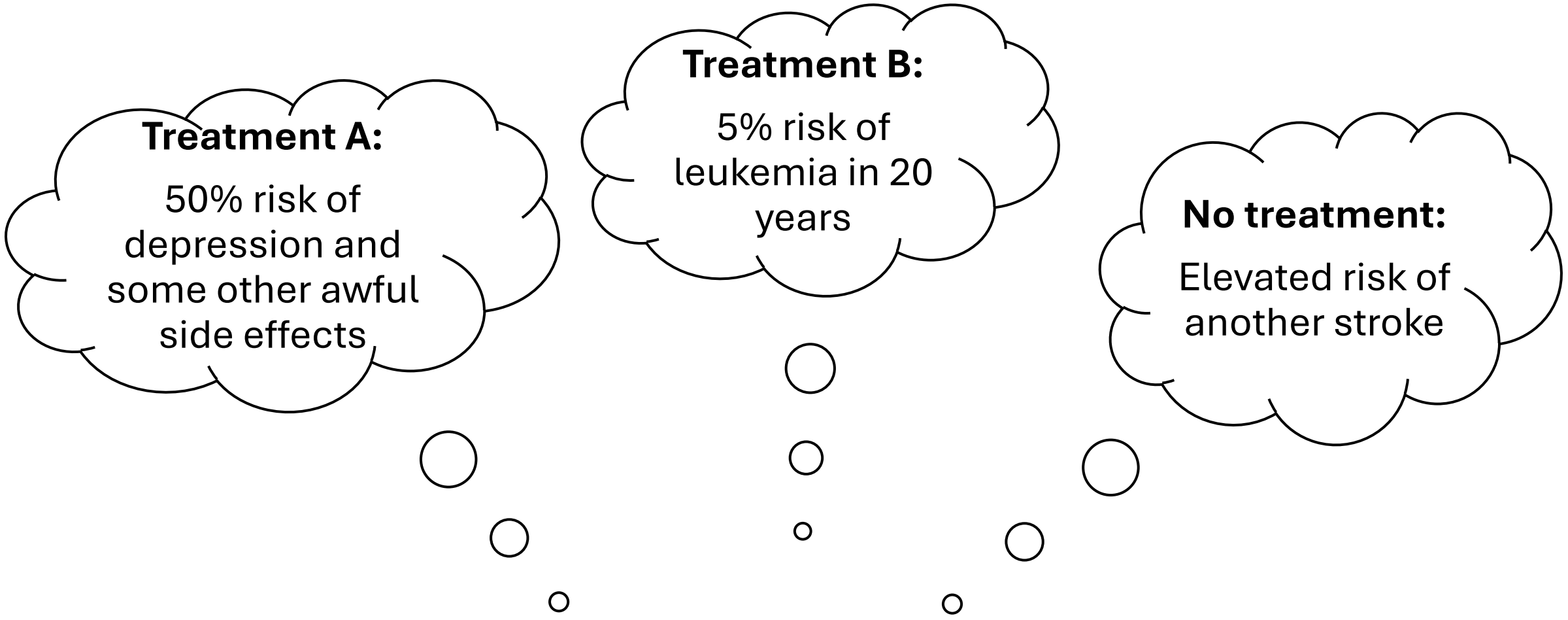
4.9.2017
STROKE

18.09.2017
BACK TO
WORK

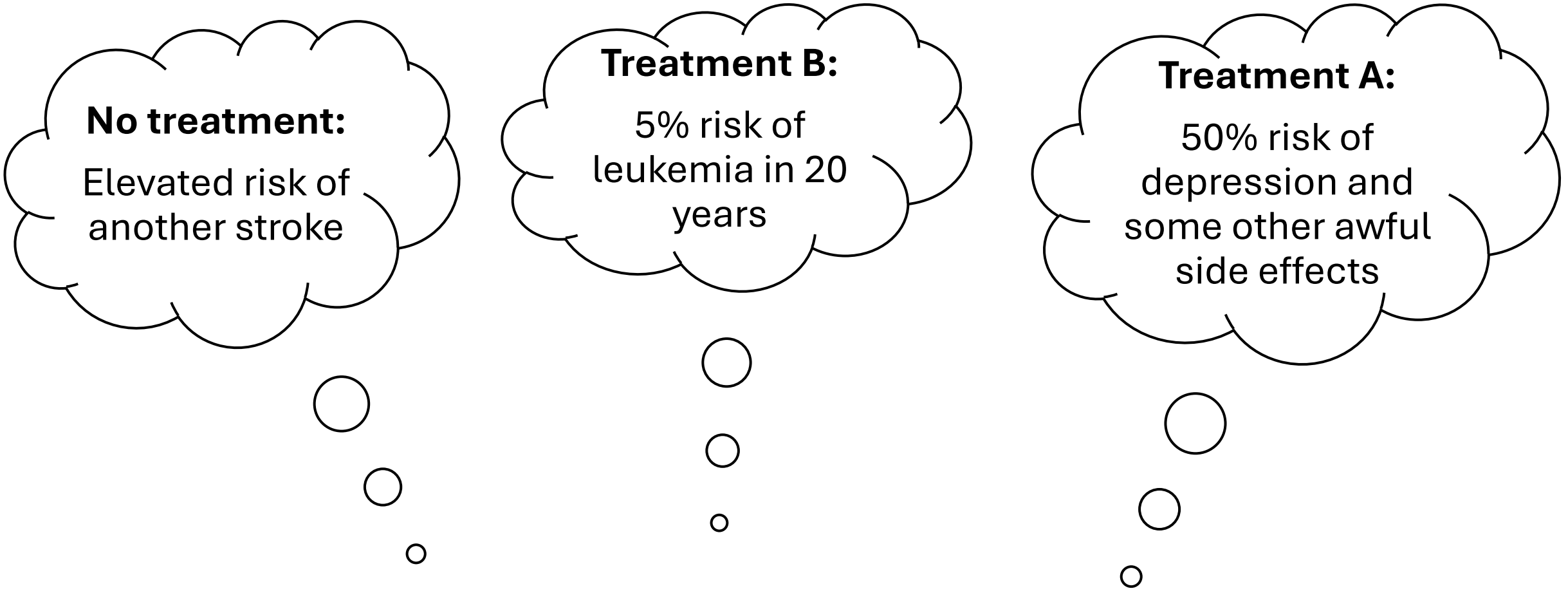
5.10.2017
CANCER
DIAGNOSIS

9.11.2017
TREATMENT
START

The difficult choice 2017



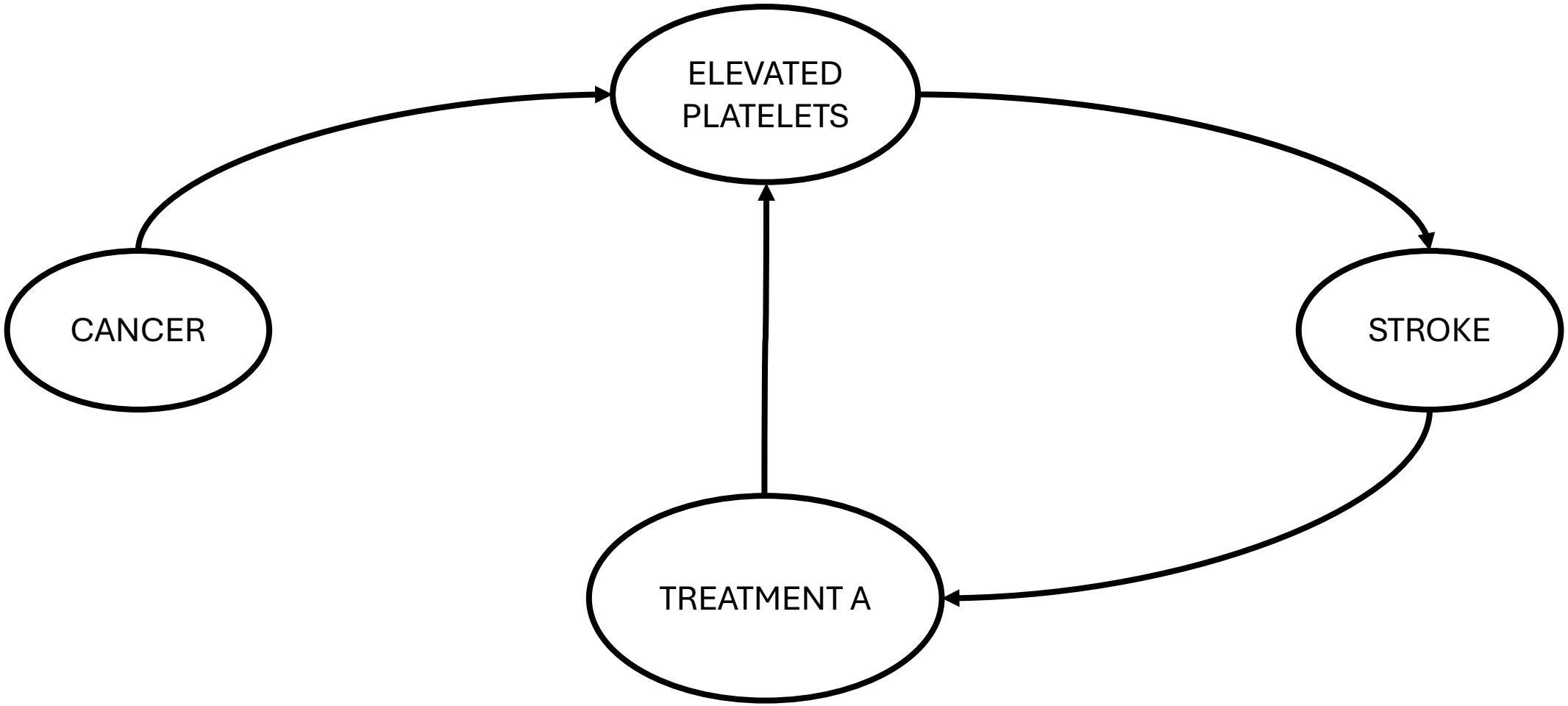
My initial preferred order



My treating phycisian’s recommendation

Final choice

Disease and treatment



My disease and my initial poor prognosis

- The official diagnosis was prefibrotic myelofibrosis(preMF)
  - Manifested in me as elevated platelets
  - Can progress to myelofibrosis
- Myelofibrosis(MF)
  - Fibrosis of bone marrow
  - At late stages the bone marrow stops producing blood cells

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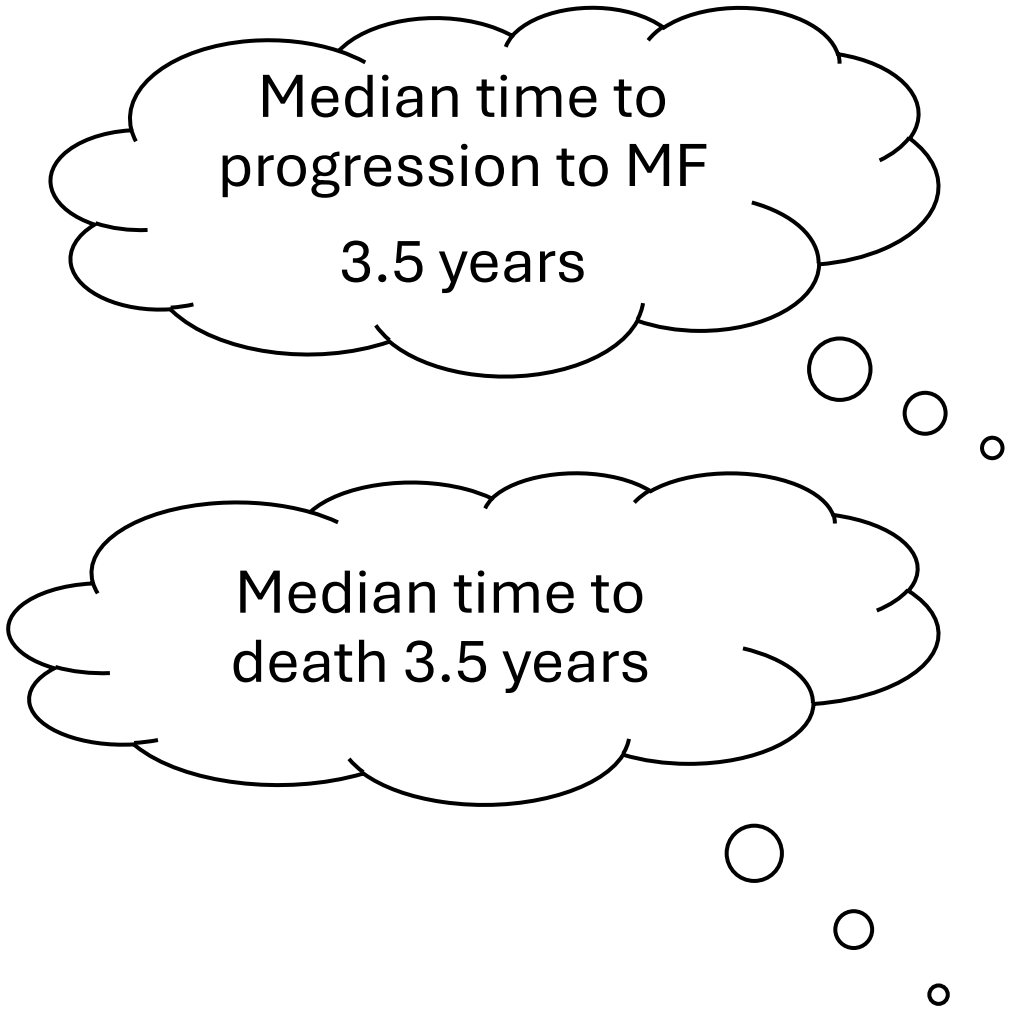
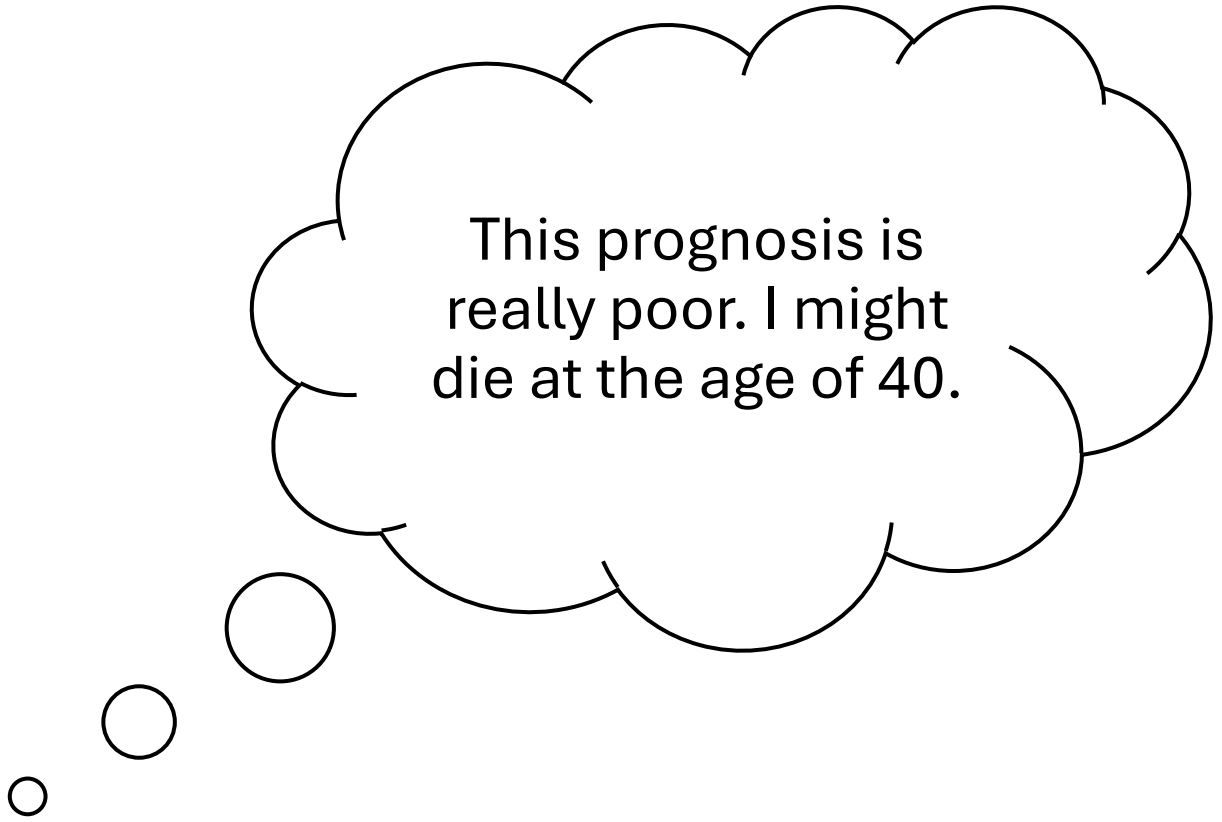
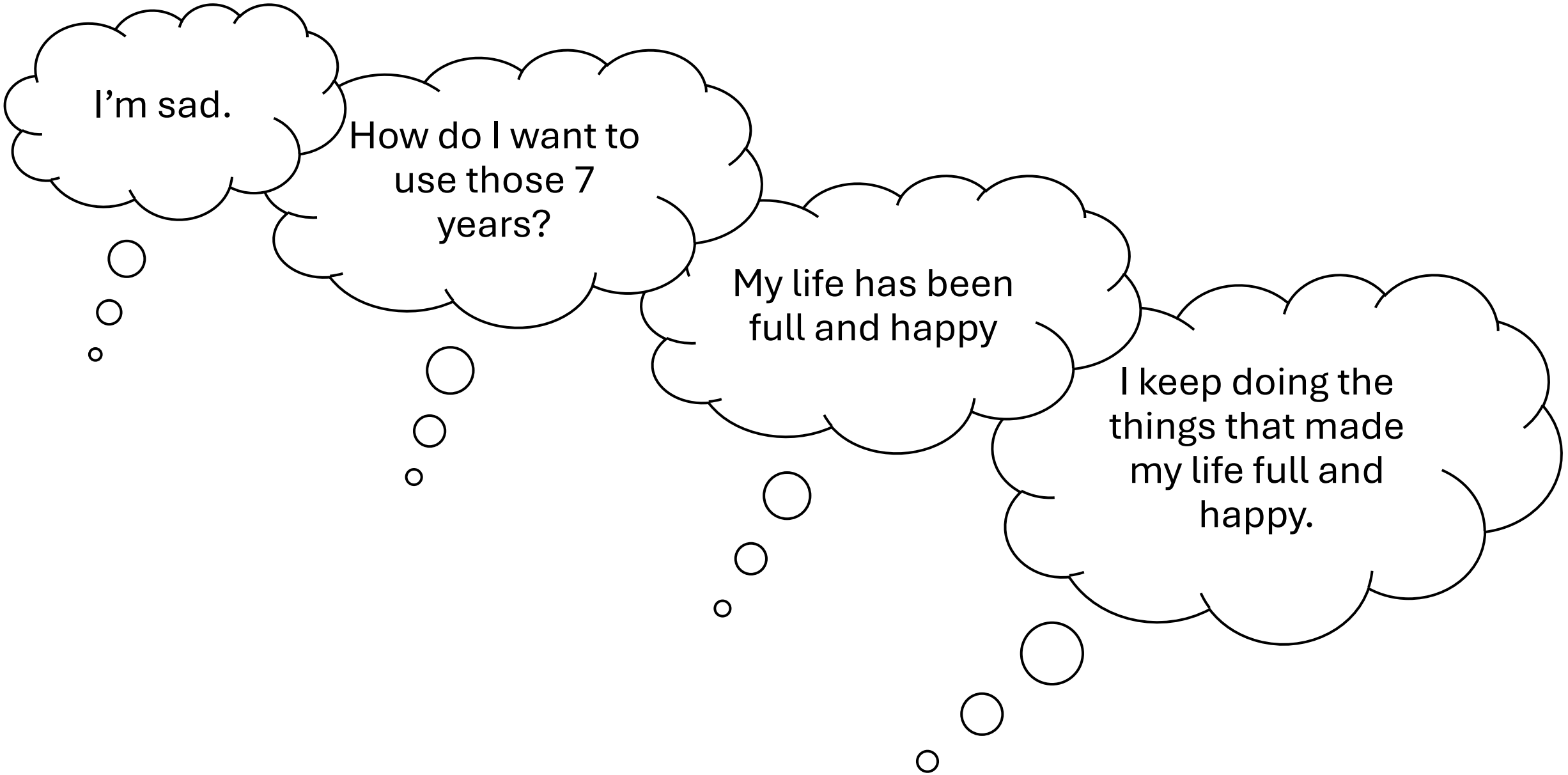


Poor prognosis – my patient perspective



Poor prognosis – my patient perspective



Poor prognosis – my statistician perspective

- Why was it poor?
  - It didn’t hold:
    - 3.5+3.5=7 estimated date of death 04 Sep 2024
  - It didn’t take into account:
    - All available medical evidence
    - My baseline characteristics
    - Current and future treatments

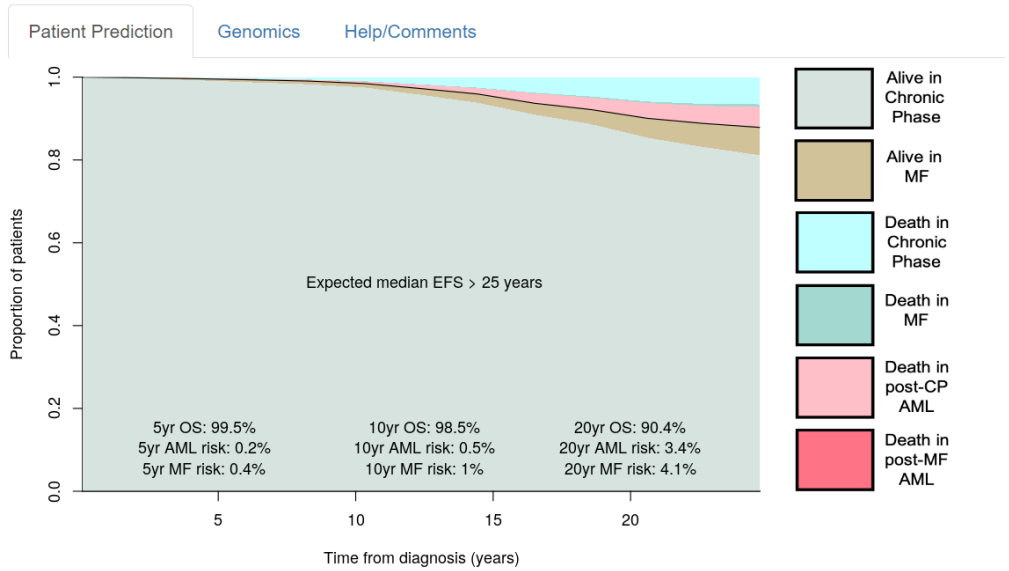
Better prognosis 2018 – RWE and shinyApp

The NEW ENGLAND JOURNAL of MEDICINE

ORIGINAL ARTICLE

Classification and Personalized Prognosis in Myeloproliferative Neoplasms

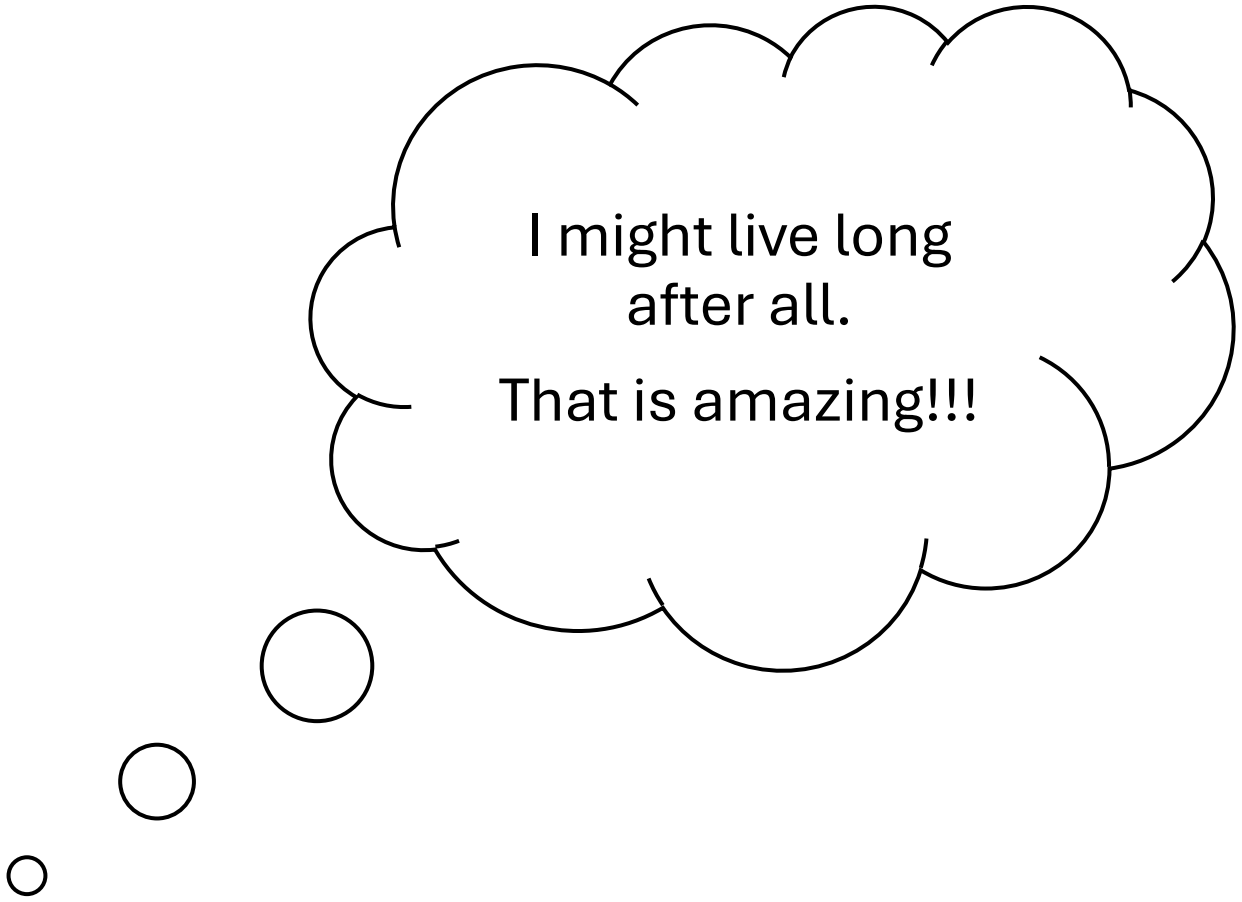
J. Grinfeld, J. Nangalia, E.J. Baxter, D.C. Wedge, N. Angelopoulos, R. Cantrill, A.L. Godfrey, E. Papaemmanuil, G. Gundem, C. MacLean, J. Cook, L. O'Neil, S. O'Meara, J.W. Teague, A.P. Butler, C.E. Massie, N. Williams, F.L. Nice, C.L. Andersen, H.C. Hasselbalch, P. Guglielmelli, M.F. McMullin, A.M. Vannucchi, C.N. Harrison, M. Gerstung, A.R. Green, and P.J. Campbell



Patient Outcomes:

Expected median EFS > 25 years

Better prognosis – Patient perspective



Better prognosis – my statistician perspective

- This is the **coolest shinyApp** I've seen
- They published it in **NEJM**!
- How did they **implement**?
- What kind of **models** did they use?
- What happens if I **change the input**?

Better prognosis – changing the input

Prior Thrombosis=YES

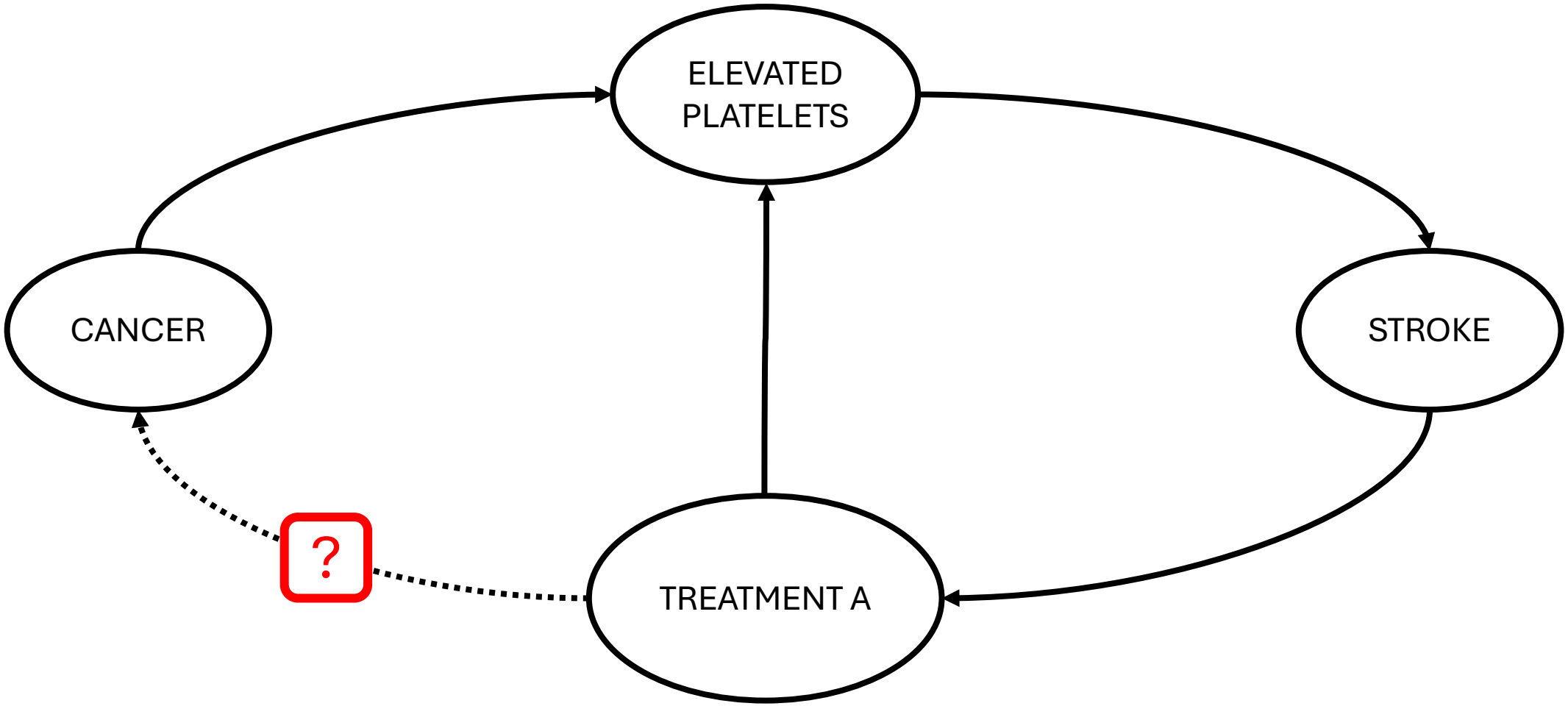
20yr OS: 90.4%
20yr AML risk: 3.4%
20yr MF risk: 4.1%

Prior Thrombosis=NO

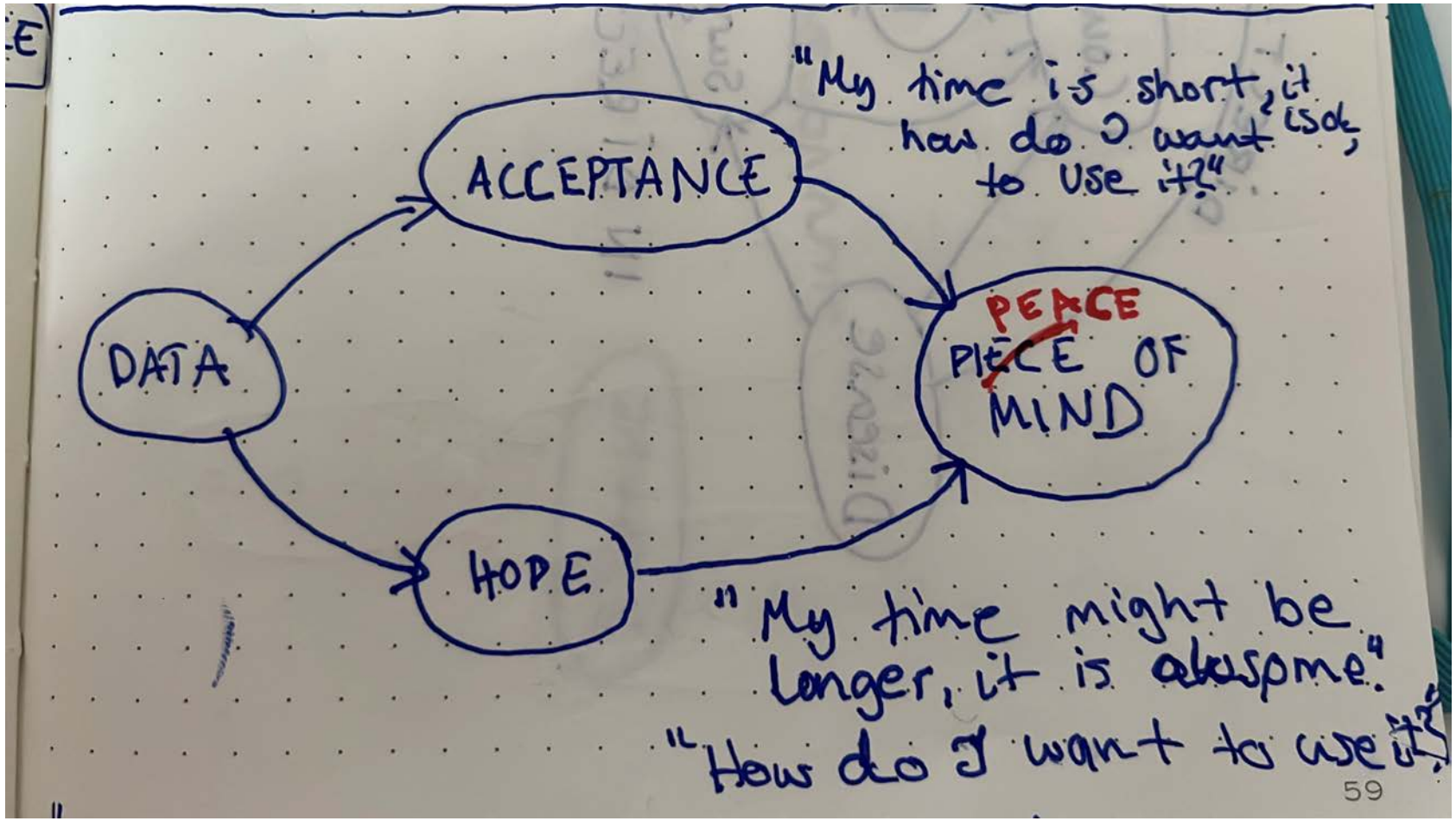
20yr OS: 88.9%
20yr AML risk: 5.3%
20yr MF risk: 5.3%

Why is the prognosis better with prior thrombosis?*
*The difference is minimal and can be due to chance

Disease and treatment – the question of my statistician mind



Making prognosis – combining perspectives



”If you can’t treat the patient you can at least tell the truth”
…and we statisticians can make an impact by data

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Patient and statistician journey

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14.12.2017
EMAIL TO
NEW
EMPLOYER

The response from my new employer

- The response was **the biggest turning point in autumn 2017**
- Why was it so impactful?
  - It **came**
  - It came **fast**, the same day
  - It was **emphatic**
  - It offered **support**
  - It **asked** what I needed and what **I and my doctor** thought
  - It **encouraged me to contact** them again if I need anything
  - It did not include **pity**, **assumptions**, or **unnecessary help**
- My advise
  - for patients: inform in **writing**, so the other person has **time to digest**
  - for leaders and colleagues: respond **timely**, with **empathy**, and **asking** what is needed

Patient and statistician journey

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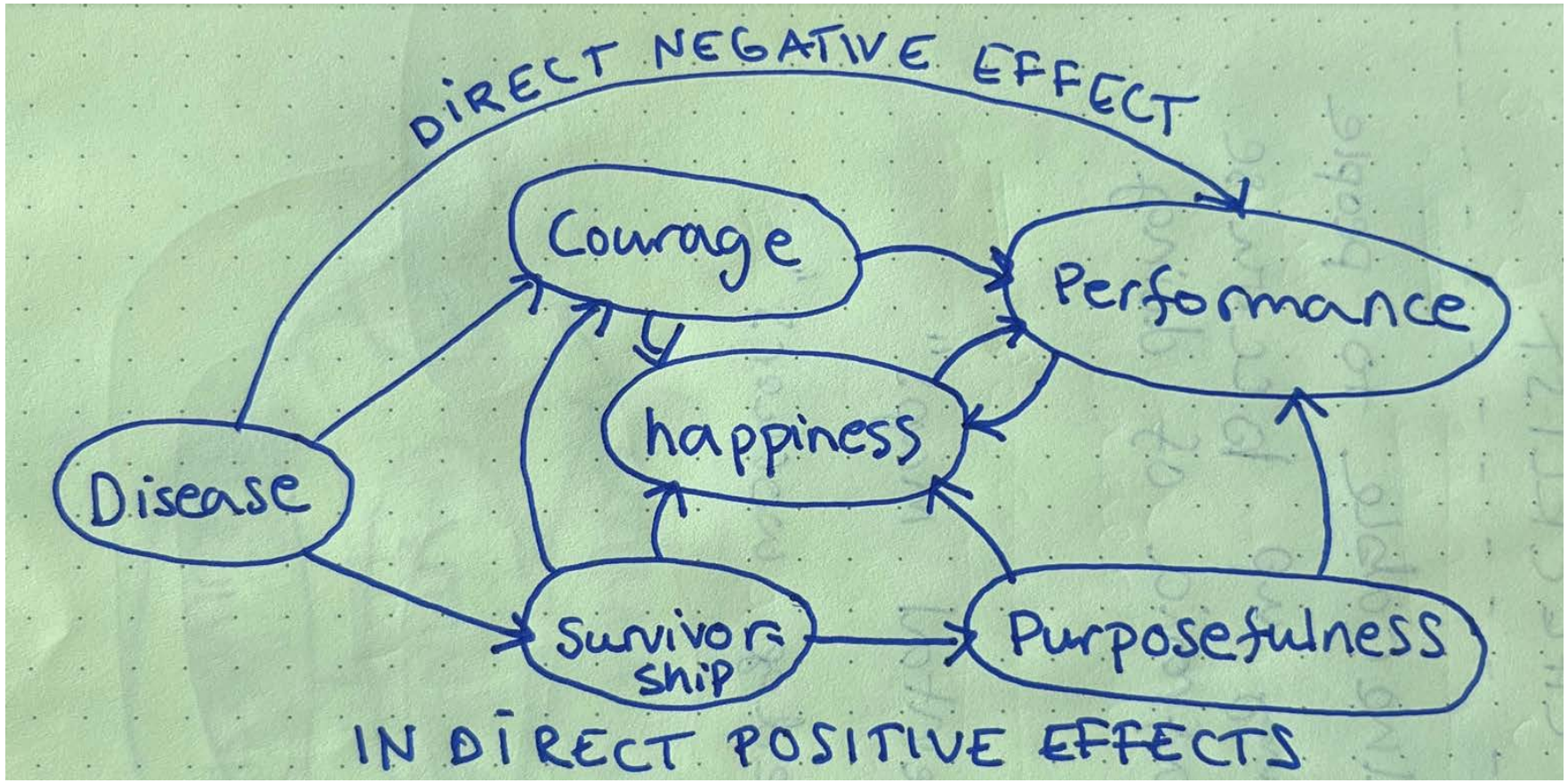
JOB
INTERVIEW

14.12.2017
EMAIL TO
NEW
EMPLOYER

3.1.2018
START OF
NEW JOB
FULL TIME

Disease and performance – My perspective



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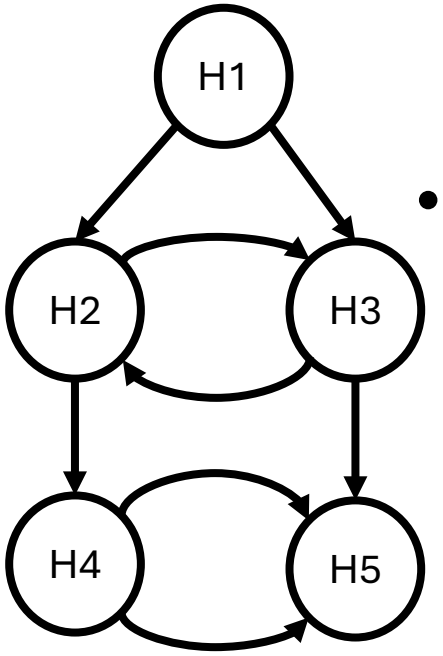
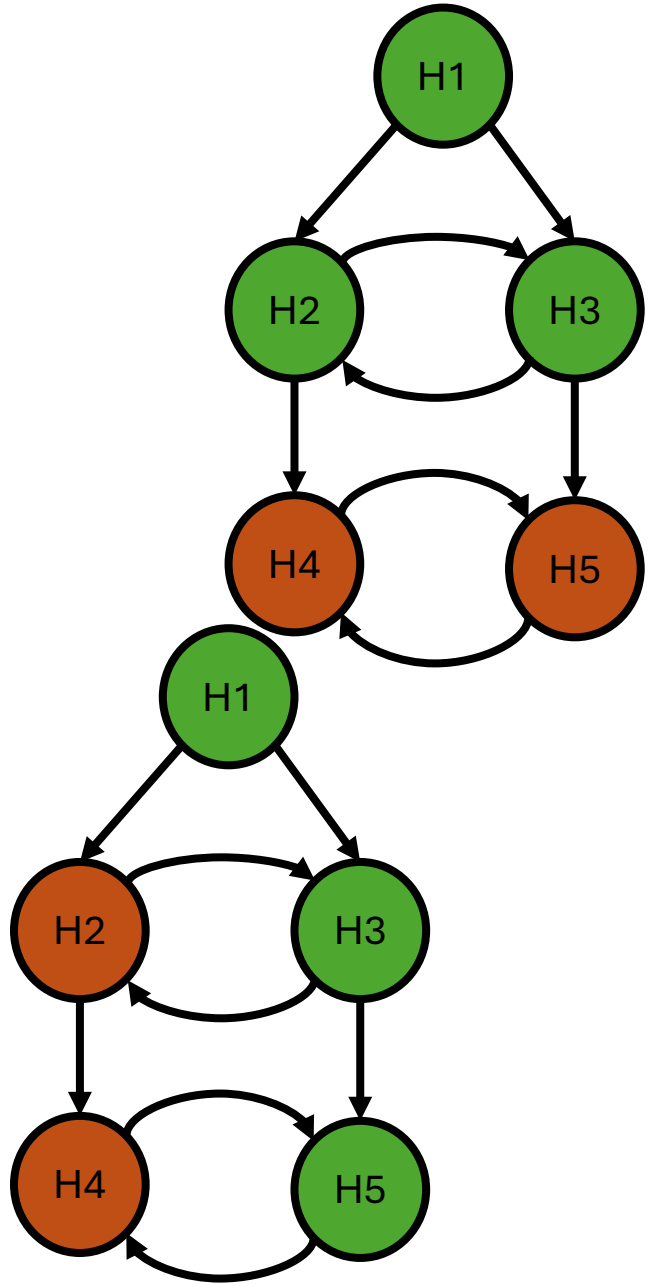
Estimands for Treatment A

- What was in the label for Treatment A?
  - Nothing for my disease, I'm on off-label treatment
- What would have been relevant for me as a patient?
  1. What is the actual risk of a given side effect?
  2. What is my efficacy if I can tolerate the treatment?
  3. Are there ways to deal with the side effects?
- Estimand for 1. and 2. is principal stratum

Type 1 error control and multipicity

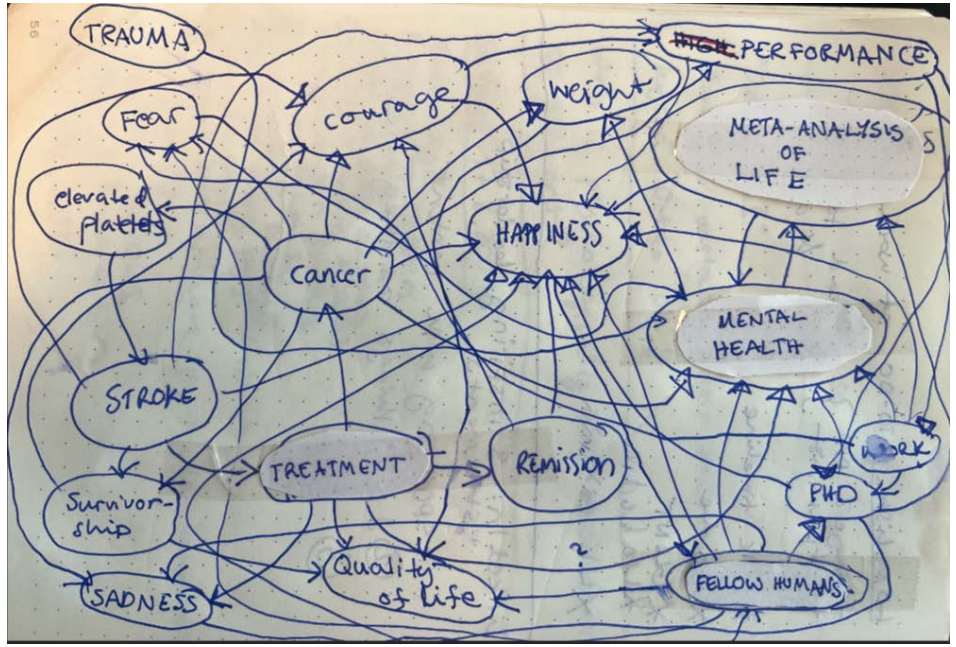


- Type 1 error control for primary endpoint and pre-spefication of secondary endpoints is important
- …however…
  - As a statistician, I would rather use my **time** on collecting data **reliably** than on optimizing and changing testing hierarchy and alpha splitting
  - As a patient:
    - I like to have the **relevant secondary endpoints** in the label
    - Unadjusted p-values are more informative than adjusted(in publications etc)

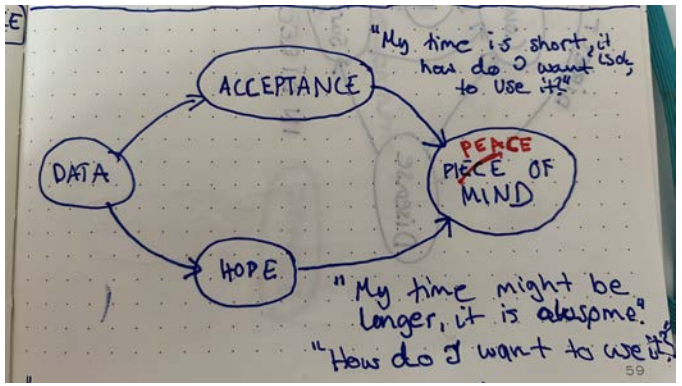


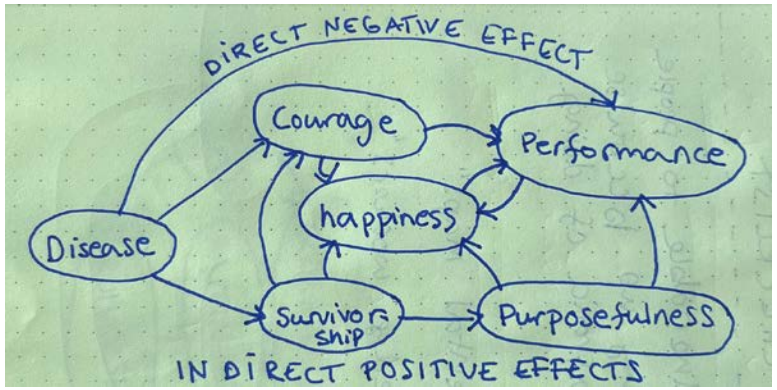
Advanced statistical methods

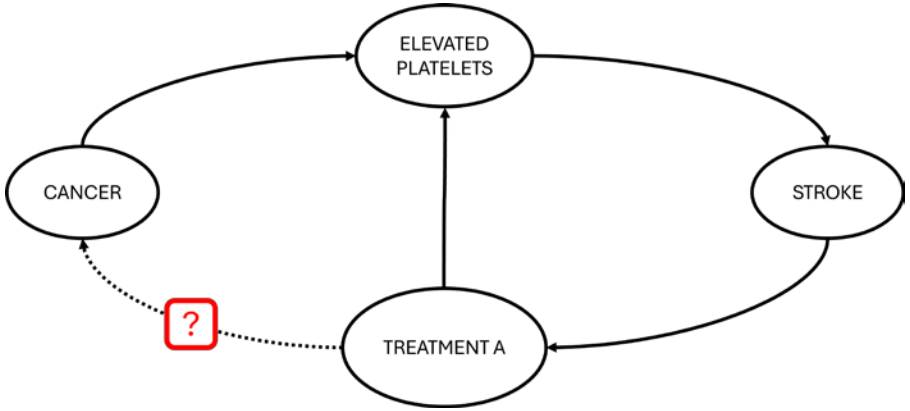
First draft for this presentation…



- Patient journeys are **complicated**
- It is good to look the total picture and try to understand all **relevant dependencies**…
- …however, answering important **subquestions reliably** is more important than being able to model everything simultaneously







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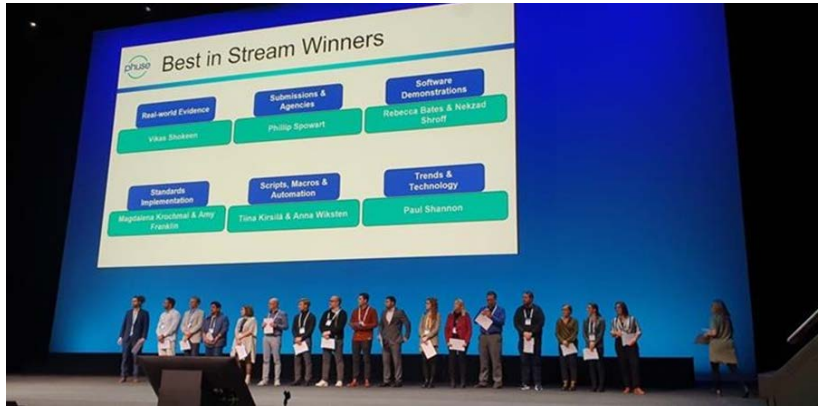
Summary

- Data is powerful on its own
- Beyond treatment we can impact patients and colleagues lives
- Patient mind is unique
  - ➢Relevant and reliable data is crucial to support the choices

Where am I now?

- Complete hematological response since 2019
- Weekly low dose injection of treatment A
- Mental health support for some other parts of life
- Living a full and happy life



”The better we do our job
the more patients we have
as colleagues”

Thank You!