Patients controlling their own Data: Motivations and Perceptions

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Background

- 2000: Retired after IPOing company I founded
  - No ordinary Schmuck, motivated + effective Schmuck
  - 4 degrees, MIT, 16th biggest IPO
- 2014: p1RCC diagnosis - Rare, Incurable, Terminal
  - Incurable, Terminal - Unfortunate, since I’m not interested in dying
  - Rare - Unfortunate since most researches aren’t looking at P1RCC

Things I tried Initially

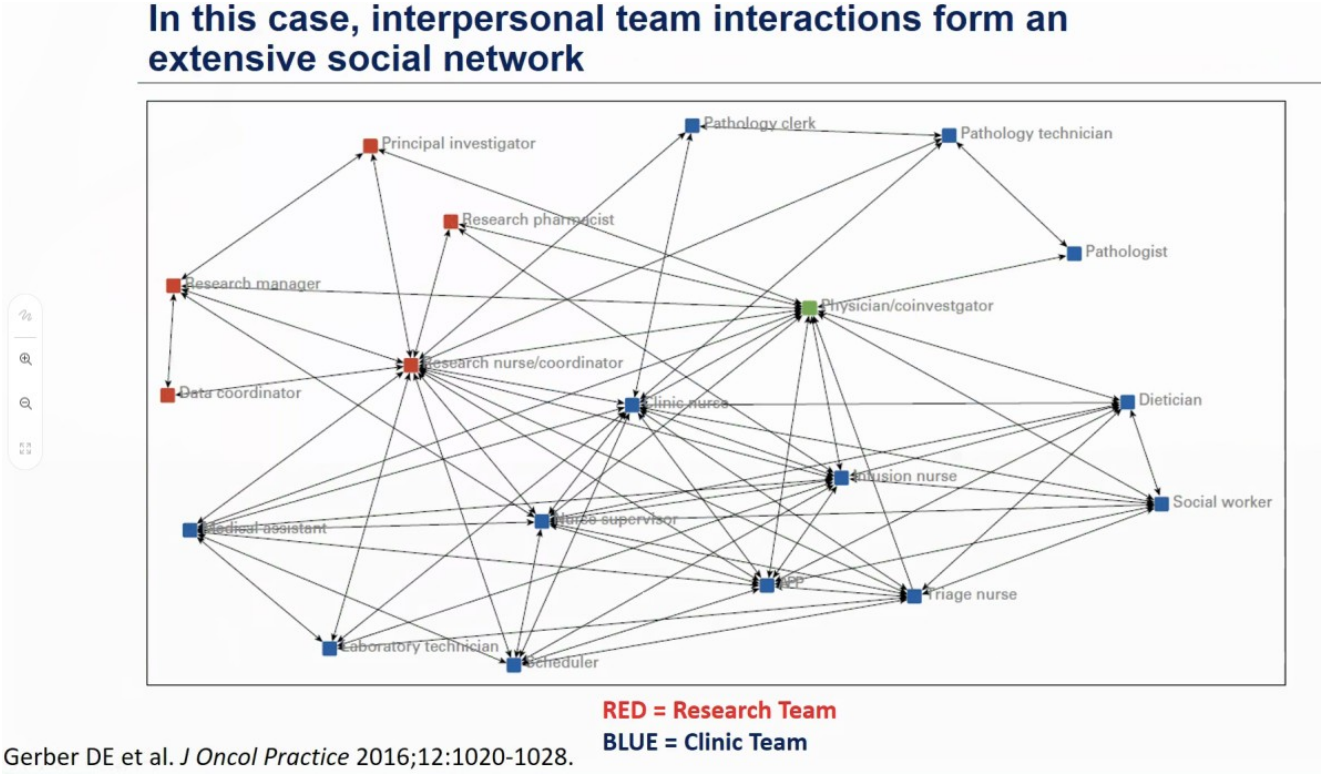
- 2015: rarekidneycancer.org
  - Goal: Get more p1RCC patients into clinical trials
- 2016: Clinical Trial tool
  - Goal: inform rare kidney cancer patients about clinical trials
- 2017: Paper: "Recommendations for the Management of Rare Kidney Cancers" in European Urology
  - Goal: inform rare kidney cancer patients about treatment options
- 2017: No increase in Overall Survival for 10 years
- In the immortal words of the FDA's Dr. Janet Woodcock: "It's not working."

https://www.latimes.com/science/story/2019-09-07/fda-medical-innovation-falls-short

Today's Talk

- Things I learned about Cancer Research
- Trying something different
- Next Steps

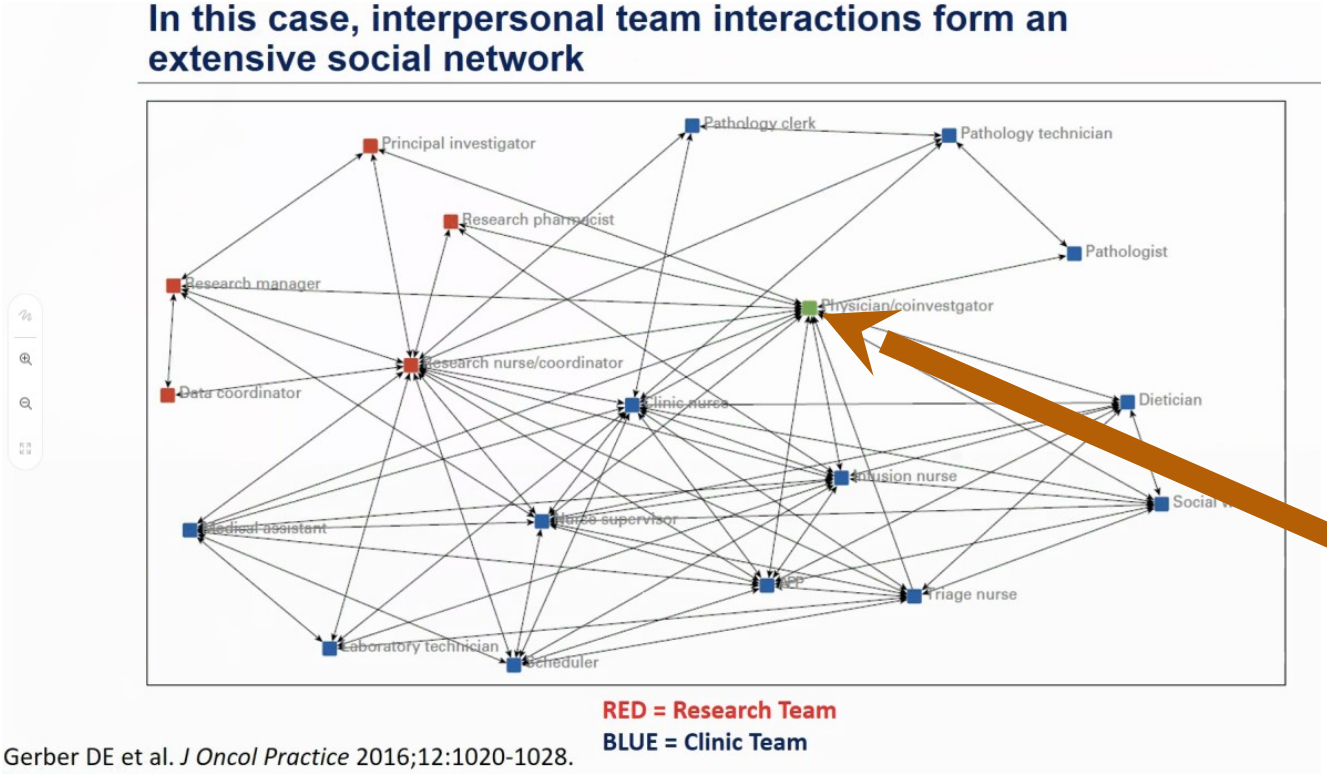
1. In a Cancer Researchers' social network...



Gerber DE et al. *J Oncol Practice* 2016;12:1020-1028.

Principal investigator
Research Coordinator
Pathologist
Physician
...

1. … **patients aren't involved** (in fact don't even exist).



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PATIENT!!

Patient Advocate (e.g. Smart Patients)

So Patients aren’t involved. So What?

- What difference could it make?

2. Researchers and Patients view time differently

- Units of Measure
  - Researchers time measurement: PFS, OS, yearly conferences and tenure tracks
  - Patients time measurement: months between treatments; months until death
- Time pressure often affects Motivation
  - Researchers are motivated to win
  - Patients are desperate to win
    - 49ers Quarterback Steve Young: in Football, you have 60 minutes
    - Last thoughts of an airline pilot in a nosedive?: They feel stupid
    - Can you do something if your life depended on it? If “yes” and you are not doing it now, it is a motivation issue, not a training issue.
- E.g. Email Responsiveness
  - Patients are more responsive than Young Researchers
  - Young Researchers are more responsive than Established Researchers

3. Cancer Research is done with **big teams**

Field	% of Papers with <4 Authors
Economics	85%
Astronomy + Astrophysics	41%
Genetics	21%
Cancer	12%

This surprised me because...

- NY Times: Can Science be too Big?
  - Big Teams confirm findings
  - Small Teams generate new ideas
  - Disruption is inversely proportional to author count
- Small teams are more agile
  - Small enables closely space milestones (speed)
  - Closely spaced milestones keep you on track

https://www.natureindex.com/news-blog/paper-authorship-goes-hyper
https://www.nytimes.com/2019/02/13/science/science-research-psychology.html

4. Cancer Researchers do a lot of the **same thing**

- Personal Genomics has been going on a long time, and it doesn't always work

This focus on the familiar surprised me because...

- Variety: If something doesn't work, it pays to do something different
  - Lots of literature: Explore/exploit; Multi-Armed Bandit; Win/Stay -- Lose/Shift
- Simultaneity: Multiple projects can be done at the same time
  - Owning more than one stock increases the chance of a home run
  - Uncorrelated business portfolio with 5% success rate leads to higher payoffs (and requires risk-tolerance)
- There are Other Things to try:
  - Cancer Vaccines (Levy Protocol – in vivo CAR-T cancercurechallenge.org)
  - Thymus research (DiGeorge Syndrome)

So how about trying something different?

Current Cancer Research Approach:

Big teams slowly doing the same thing without any patient involvement.

Silicon Valley Approach

Small teams quickly doing different things while including the patient.

Something different: 2018 SV.ai Hackathon

- Patient Centric, Small Teams, (very) short TimeFrame
- Pete Kane (Pete@sv.ai) – 200 researchers → 150 researchers
- Steve Tamm (SalesForce CTO) – Venue
- Bill (Rarekidneycancer.org): co-ordinated sample, data processing ...
- Max Meng/Tasha Lea (UCSF) expedited and paid for BGI DNA-seq
- Continuity
  - QuantumInsights.io investment led to Dr. Feltus (Clemson)
  - Alex and Reed Bender (Clemson) have done work since the hackathon
    - Kaya Bilguvar, Christopher Castaldi (Yale) has recently done RNA-seq
      - Mike D'Amour

Benefits from controlling my own data: WNL detector

- First Benefit of being able to access my own data
- 20190129 - Redemonstrated thoracic aortic ectasia, measuring up to 4.3 cm in maximal diameter, unchanged since 11/7/2017 ← Never measured.
- Eric Topal: “Within Normal Limits” → “We Never Looked”
- Real data: 20140605 - Stable dilated ascending aorta measuring up to 4.1 cm
  - After nephrectomy
- Stability over 4.5 years is better than stability over 1.5 years,
- Cardiac Health is an issue with Cancer Survivors
- More likely to die from an aneurism than cancer?

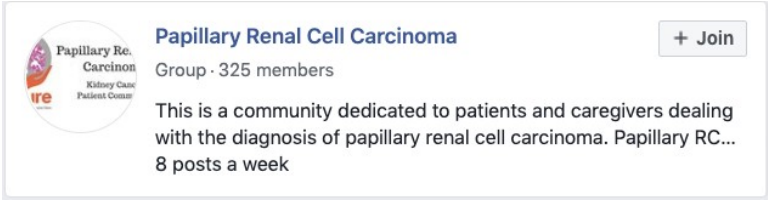
Next Steps: I’m looking for partners.

- So, now that I’ve alienated 95% of the audience….
- I’m interested in establishing partnerships with the 5% who want to move in this direction
- If you’re interested in trying something new, and working on a timeframe that better serves patients, please contact bill@rarekidneycancer.org

RareKidneyCancer.org

Reliable Information about Rare Kidney Cancers

Kccure.org



RESEARCH TO THE PEOPLE.

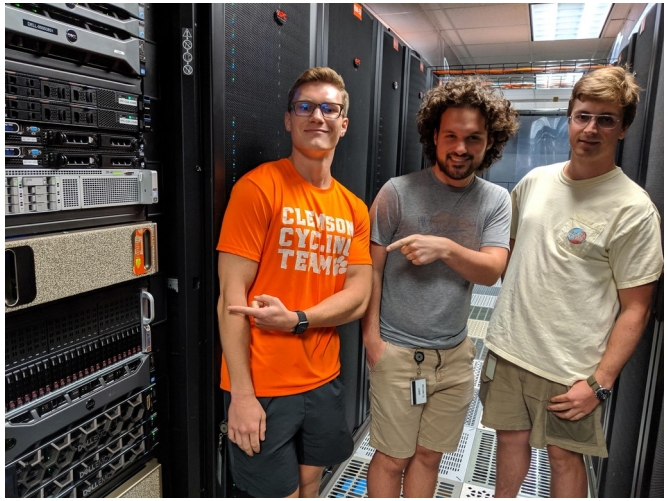
Formerly sv.ai

Small teams of young researchers

Reed Bender
Bioengineering, B.S.

How did I get involved?

- The Systems Genetics lab at Clemson
  - Small team led by Dr. Alex Feltus
  - Diverse educational fields
  - Diverse age ranges (Lower class Undergraduates to Postdocs)
- Silicon Valley Approach
  - Lab consists of:
    - Geneticists
    - Bioengineers
    - Computer Scientists
    - Computer Engineers
    - Biochemists



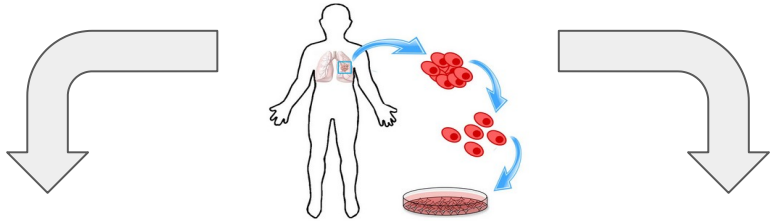
Perfect combination of...

1. An intelligent patient
    - Obtained his own genetic data
    - Knew **how** to share it

2. A motivated research collaborator
    - Bill Paseman found **who** to share the data with
    - Young student collaborators with motivation and interest

What did we do different?

- Took the standard of cancer genomics research, and developed a process to make it relevant to **patients**
  - The Cancer Genome Atlas (TCGA)
  - The Genotype-Tissue Expression Project (GTEx)



NATIONAL CANCER INSTITUTE
THE CANCER GENOME ATLAS

GTEx Portal
"Viewing Gene Expression Data on the GTEx Portal"
BROAD INSTITUTE

Ultimate Goals

- Find more **patients** like Bill Paseman to obtain and share data
- Engage more **students** in patient-centered research

For more information on the research outcomes, visit the poster session:

The Paseman Process: A Patient-Centered Approach to RNAseq

Spares