How to Work with Patient Representatives to Make Your Research More Successful

SITC Cancer Immunotherapy
Winter School

January 17, 2020

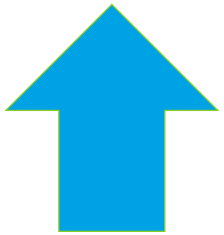
Deborah Collyar

PAIR: Patient Advocates In Research

Which statement is correct?

A. “the patient failed the treatment”

B. “the treatment failed the patient”



Please **stop** using this one!

Time to change language that blames patients…

e.g., “response” and “non-responders”

Patients respond to treatment!

Their disease may not

Help us change regulations, mindsets, & practice

Let’s work together on **accurate** language

What business are you in?

Immunotherapy?

Only?

How does it fit?

Oncology?

What kind?

When?

Science/research?

For whom?

How does it connect?

PEOPLE business!

The U.S. ~~healthcare~~ disease crisis system

Patients are PEOPLE

Who just landed on a new planet with:

- No roadmap
- No dictionary
- No survival training

Why do clinical trials exist?

People need better treatments… but not at all costs

Issues start with:

- (mis) Diagnosis
- Confusion at each step
- Technology for ‘big data,’ not patient results
- Costs (many kinds)
- Clinical trials?

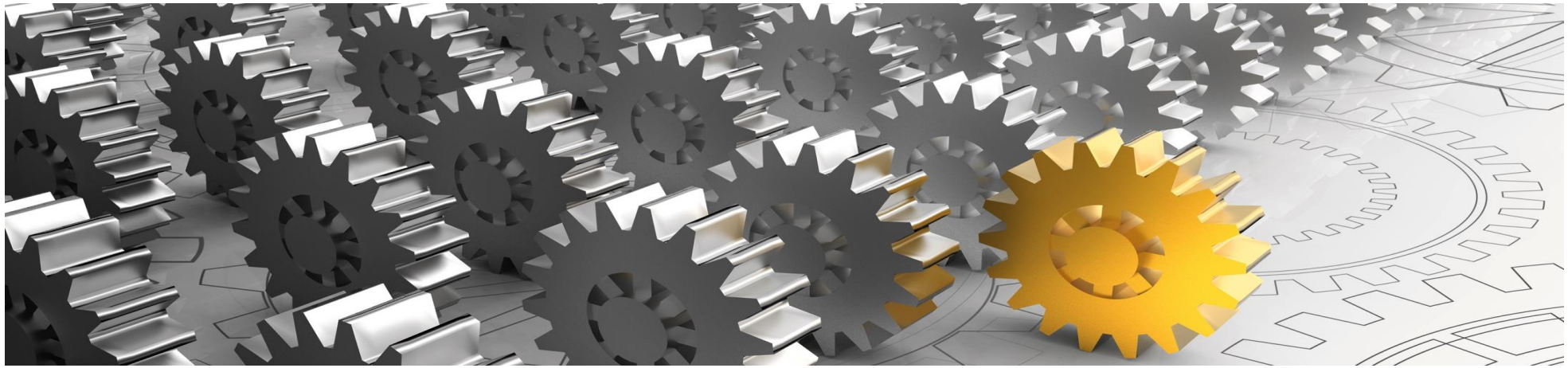
What do patients want?

Patients want to be PEOPLE again.

BETTER, not just more treatments.

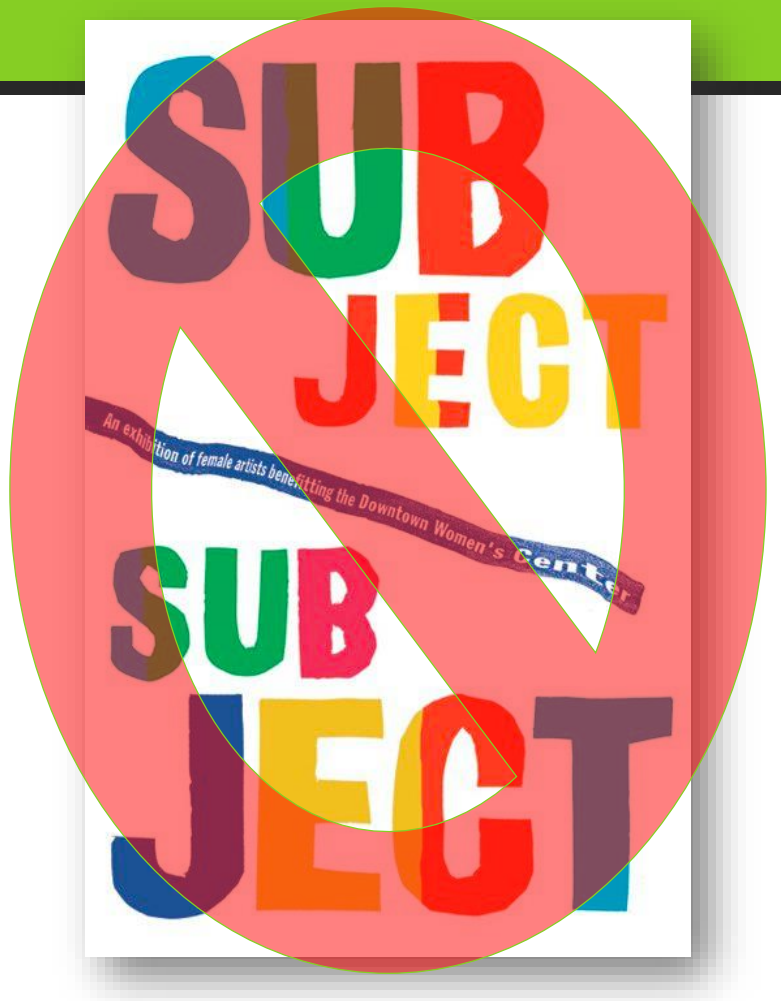
Answers they can use (ct.gov?)

Answers that work for them, not just others.



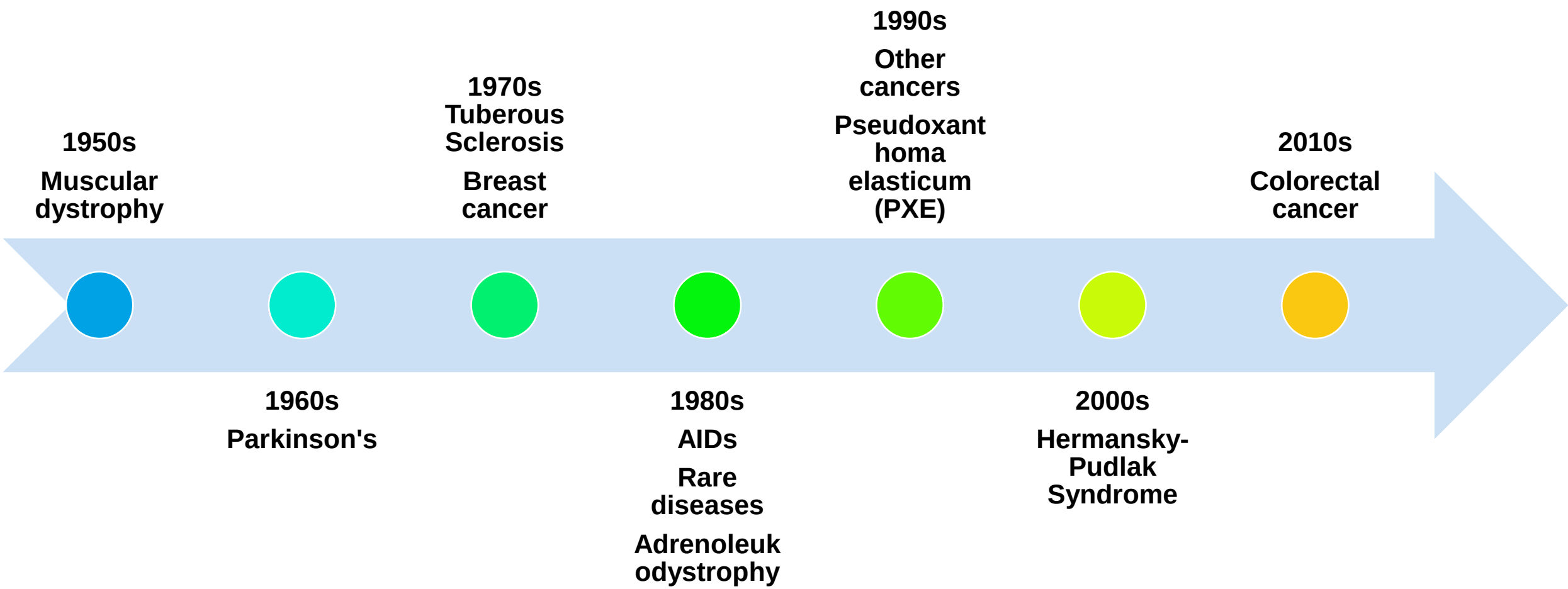
What do patients want to know?

- ✓ I am not alone (others before me)
- ✓ Why are you doing it?
  - What is known/unknown
- ✓ What to expect
  - Exploratory v. validated
- ✓ How bad can it get… 'safe' word?
- ✓ What happens after?



MORE THAN INFORMED CONSENT

Patients & Advocates have influenced research for decades, e.g.



A few examples…

CMTP

contact us newsletter signup

about us our work news room green park collaborative resource center

Home › Resource Center › New Guidance for Treatment Switching in…

Best Practices for the Design, Implementation, Analysis, and Reporting of Oncology Trials with High Rates of Treatment Switching

GPC released a report that provides guidance for researchers who lead oncology drug trials that Download

SCIENCE AND SOCIETY

How have patient advocates in the United States benefited cancer research?

Deborah Collyar

NATURE REVIEWS | CANCER VOLUME 5 | JANUARY 2005 | 73

Outcomes and endpoints in cancer trials: bridging the divide

CrossMark

Michelle K Wilson, Deborah Collyar, Diana T Chingos, Michael Friedlander, Tony W Ho, Katherine Karakasis, Stan Kaye, Mahesh K B Parmar, Matthew R Sydes, Ian F Tannock, Amit M Oza

www.thelancet.com/oncology Vol 16 January 2015

Cancer is not one disease. Outcomes and endpoints in trials should incorporate the therapeutic modality and cancer type because these factors affect clinician and patient expectations. In this Review, we discuss how to: define the importance of endpoints; make endpoints understandable to patients; improve the use of patient-reported outcomes; advance endpoints to parallel changes in trial design and therapeutic interventions; and integrate these improvements into trials and practice. Endpoints need to reflect benefit to patients, and show that changes in tumour size either in progression) or relative to control (progression) are clinically relevant. Improvements npanied by improvements in available endpoints. Stakeholders need to come together h for research that ensures accountability and optimises the use of available resources.

Lancet Oncol 2015; 16: e43–52

University of Toronto Princess Margaret Cancer Centre, Toronto, ON, Canada (M K Wilson FRACP, Prof I F Tannock PhD, Prof A M Oza FRCP, K Karakasis MSc); Patient Advocates In Research, Danville, CA, USA (D Collyar BSc); The Noreen

VOLUME 34 · NUMBER 10 · APRIL 1, 2016

JOURNAL OF CLINICAL ONCOLOGY ASCO SPECIAL ARTICLE

Use of Biomarkers to Guide Decisions on Adjuvant Systemic Therapy for Women With Early-Stage Invasive Breast Cancer: American Society of Clinical Oncology Clinical Practice Guideline

Lyndsay N. Harris, Nofisat Ismaila, Lisa M. McShane, Fabrice Andre, Deborah E. Collyar, Ana M. Gonzalez-Angulo, Elizabeth H. Hammond, Nicole M. Kuderer, Minetta C. Liu, Robert G. Mennel, Catherine Van Poznak, Robert C. Bast, and Daniel F. Hayes

Cell

Leading Edge

Commentary

Core Clinical Data Elements for Cancer Genomic Repositories: A Multi-stakeholder Consensus

Robert B. Conley,[1] Dane Dickson,[2,*] Jean Claude Zenklusen,[3] Jennifer Al Naber,[1] Donna A. Messner,[1] Ajlan Atasoy,[4] Lena Chaihorsky,[5] Deborah Collyar,[6] Carolyn Compton,[7] Martin Ferguson,[3] Sean Khozin,[8] Roger D. Klein,[9] Sri Kotte,[10] Razelle Kurzrock,[11] C. Jimmy Lin,[10] Frank Liu,[12] Ingrid Marino,[13] Robert McDonough,[14] Amy McNeal,[15] Vincent Miller,[13] Richard L. Schilsky,[16] and Lisa I. Wang[17]

NCI SPORE
Patient Advocate Research Teams (PART)

Planning

Direction

Decisions

Discovery

Observation

Proof of concept

Development

Preclinical

Tissue + trials

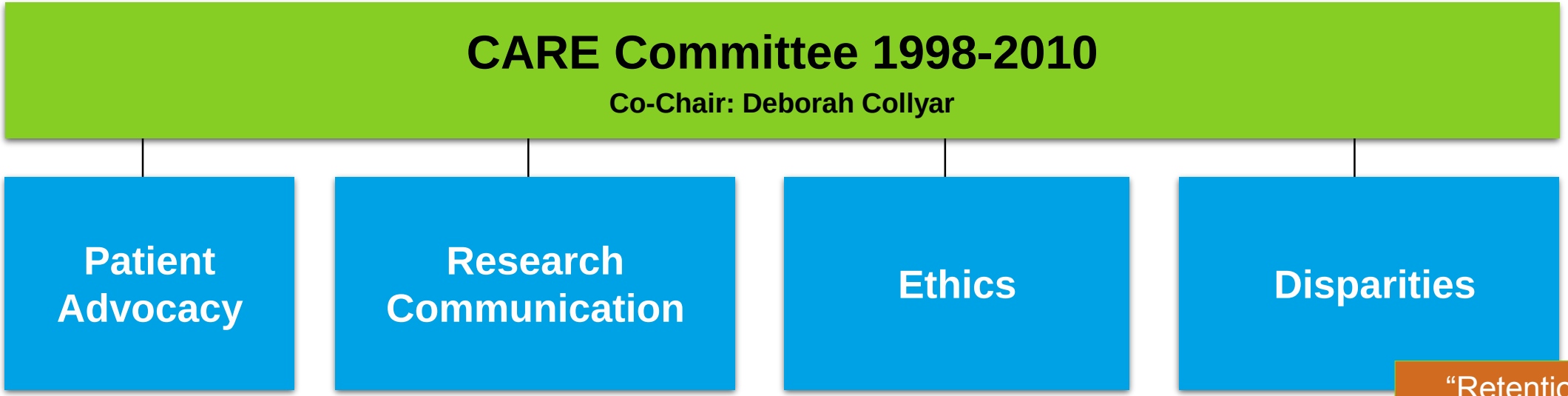
Barriers

Intellectual Property

Interoperability

Collyar D, Nature Reviews Cancer 2005

e.g. Cancer & Leukemia Group B



Themes: Service, educational, and research activities

Development → **Approval** → **Activation** → **Recruitment** → **Endurance** → **Results**

Reviews:
Operational
Concept
Protocol

Informed consent:
Templates
Lexicons

Recruitment plan:
Tools
Special populations

Tracking and advice:
Resource networks
Protocol evaluation
Accrual Plan adjustments

Participant communication:
Thank you letters
Research summaries

Recruitment plans help

Patients

Situation, influencers, needs, preferences

Sites

Logistics, barriers, communication tips

Referrals

Awareness, inclusion, positioning

http://bit.ly/2Pfiv4E

Remember that roadmap idea?

Details

What is my condition/disease?

- General information exists
- Details usually don't

Experts

Who do I talk to?

- How many specialists?
- Where do I find them? Who coordinates my care?

Options

What can I do?

- For my condition/disease? Clinical trials?
- How will this impact my lifestyle?

Trials

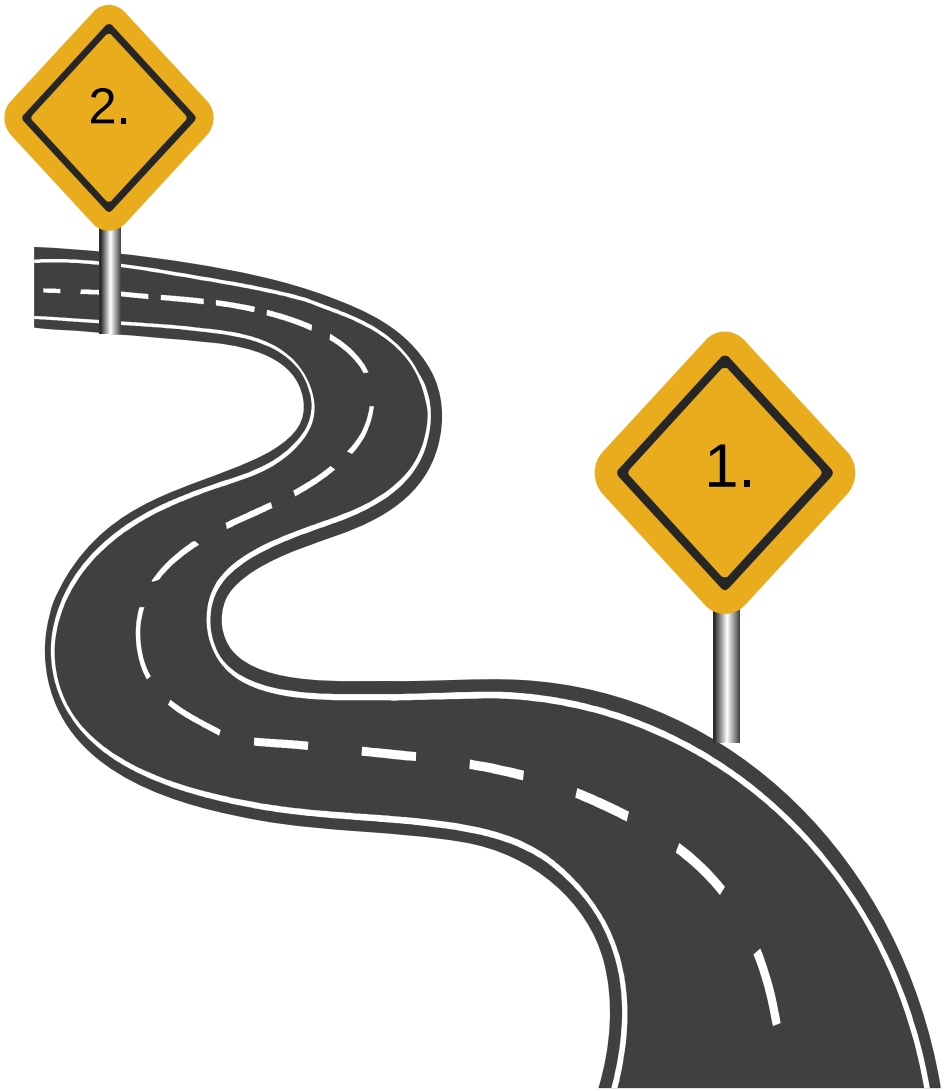
Are there clinical trials for ME?

- No matter the sponsor
- What should I expect?

Cost

How much will this take?

- For treatment, tests, checkups, other care?
- Initially? Ongoing?



What ***should*** clinical trials really be about?

Re-think traditional phases

Design & conduct with ***clinical use*** in mind

Connect trials & data sharing

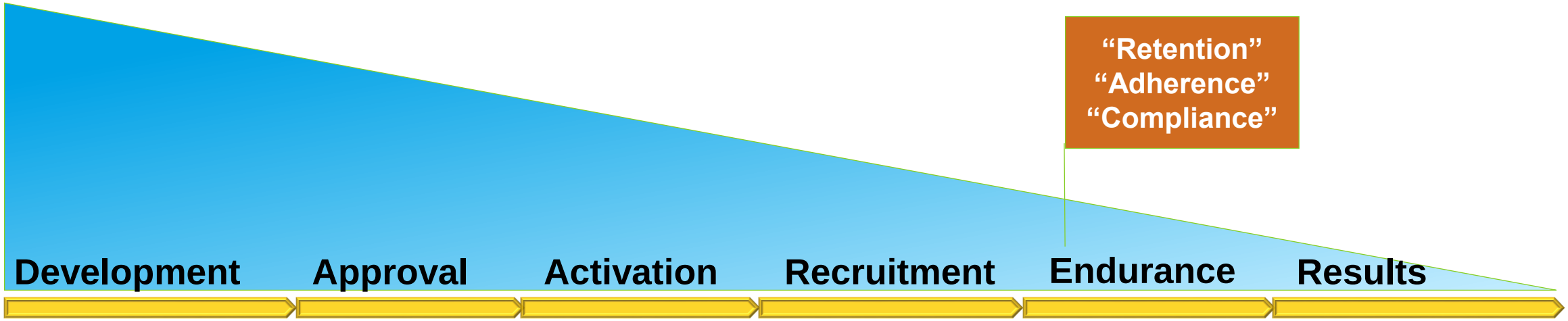
Connect modalities with immunotherapies

Technology for patient results, not ‘big data’

PROs = more than AEs

Let’s make patient-centered **change** happen!

“Patient value” in research & clinical trials



Development Plan

- Identify question
- Preclinical considerations
- Assist with trial design
- Co-Investigators

Trial Development

- Adaptive design
- Detect Issues
- Broaden eligibility
- Review informed consent

Ongoing Study

- Refine recruitment steps
- Spot retention issues
- Reduce amendments

End of Study

- Update patients on study
- Present study results
- Ensure understandability

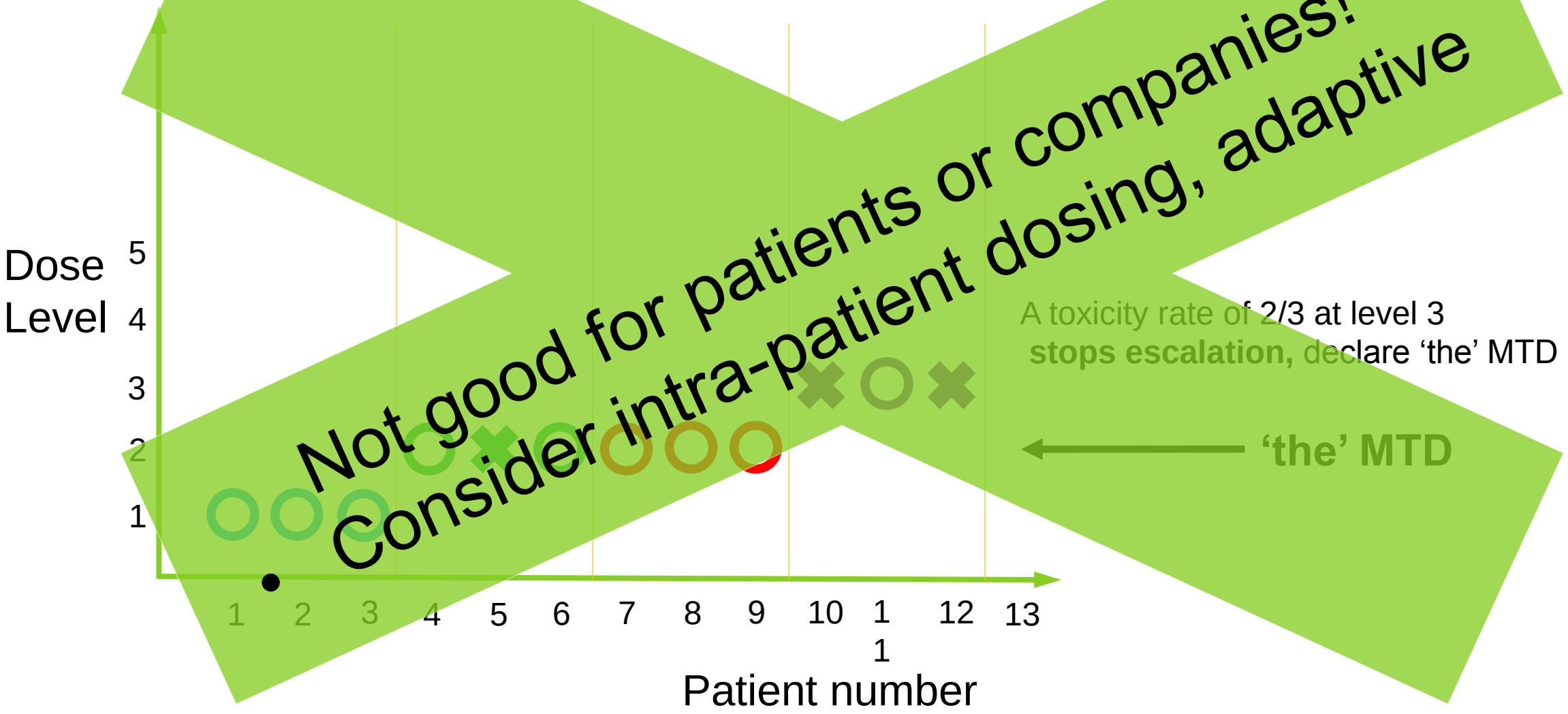
Spears P. Collyar D.https://www.diaglobal.org/_GlobalForum/2017/Sept2017/index.html

"Patient" Contributions

	Development Plan	Trial Development	Ongoing Study	End of Study
	◆ Identify the question ◆ Assist with design ◆ Be co-Investigators	◆ Detect recruitment issues ◆ Broaden eligibility criteria ◆ Review informed consent	◆ Refine recruitment steps ◆ Spot retention issues ◆ Reduce amendments	◆ Update patients on study ◆ Present study results ◆ Ensure understandability
Personal — **Patient**	◆ Relay their personal experience with disease ◆ Add a sense of urgency and need to accelerate research ◆ Give advise on potential recruitment and retention barriers, based on their personal experience ◆ Fill out Patient-Reported Outcome (PRO) instruments			
Disease — **Patient Group/ Organization (PG)**	◆ Provide broad information about the patient community/communities they serve ◆ Provide access to patients to share their personal experience ◆ Give advise on potential recruitment and retention barriers for their patient community ◆ Distribute clinical trial information to aid recruitment ◆ Distribute written public summaries of completed clinical trials to their patient communities			
Clinical Trials, Disease & People — **Research Patient Advocate (RPA)**	◆ Relay detailed understanding of patient populations, as well as their own personal experiences ◆ Provide solutions to accelerate research through collaborations and breaking down road blocks ◆ Work closely with research team as a co-investigator and give feedback about appropriate design • e.g., study endpoints, Bayesian methods, crossover, PRO and other trial considerations ◆ Address recruitment/retention for each patient population and environment ◆ Help write and review informed consent documents for readability and understanding by patients ◆ Advise and adjust potential recruitment and retention strategies for each patient community ◆ Participate as members of data safety monitoring boards, IRBs, advisory boards, etc. ◆ Assist with written public summaries of completed clinical trials and research programs ◆ Write articles for wide public distribution about research and study questions ◆ Help present study information in public and scientific forums			

Spears P. Collyar D. https://www.diaglobal.org/_GlobalForum/2017/Sept2017/index.html

Ex: Standard phase I 3+3 dose *escalation*



Not good for patients or companies!
Consider intra-patient dosing, adaptive

Adaptive Design in Clinical Trials – Happy 50th!

≥ 1 decision point(s) in superiority trial design since 1969

KEY: Careful planning upfront

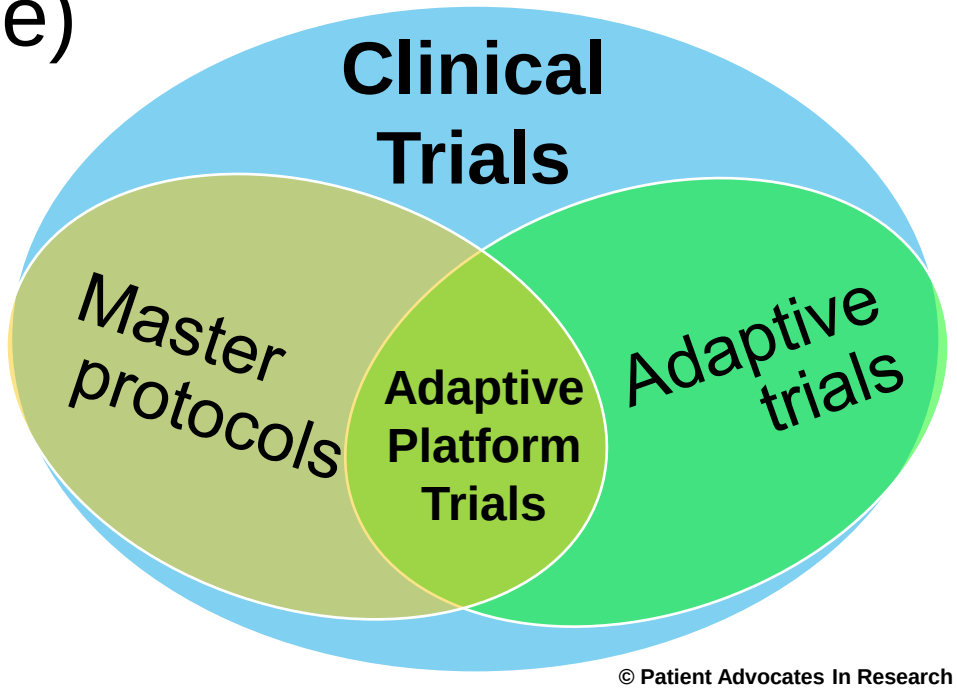
- Decision pre-specified in protocol
  - Interim analysis by DMC
  - Preserve type 1 error techniques
  - Pre-specified futility boundaries
  - Sample size adjustment

Ellenberg S. 2007 NCPF presentation

Zelen M. Play the winner rule and the controlled clinical trial. J Am Stat Assoc. 1969;64:131–146.

Why sponsors & researchers need novel designs

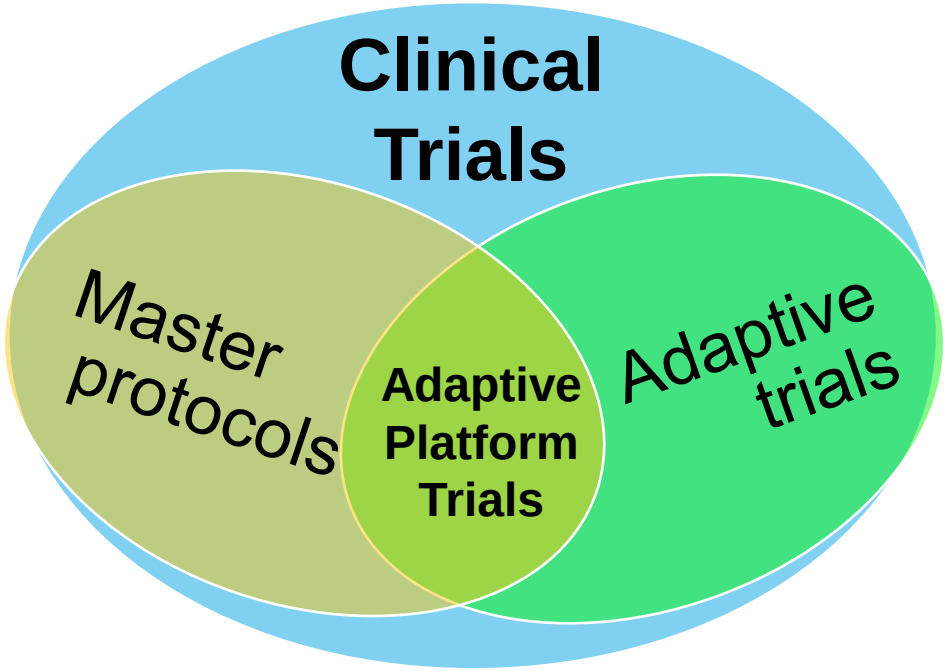
- More attention to trial success (internal & external planning)
- Identification of possible ‘intermediate endpoints’
- Faster go/no go decisions for agents
- More accurate sample size (possible)
- Include patient needs into design
- Faster identification of market
- Plan for recruitment, retention
- Adapt, share updates



https://go.nature.com/2UHyYPQ

Why novel designs make sense to patients

- Tailored to sub-type
- Better chance for ‘new’
- Contributions matter more
- Looks like they care about me
- Science learns & shares knowledge



Closer to
“A trial for every patient”

https://go.nature.com/2UHyYPQ

Regarding immunotherapy…

The “latest greatest”

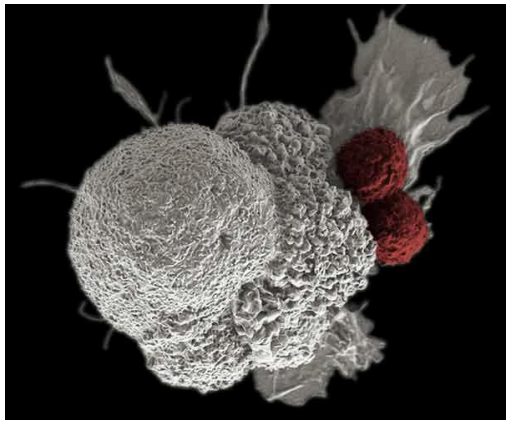
Few cancer patients are treated with IO

- Most still get surgery, radiation, and/or chemotherapy/biologicals

Immunotherapy promising, but…

- Many tumors don’t respond
- Not a replacement therapy
- Side effects
- Trial results don’t often transfer to commercial use

Please set ***reasonable expectations***!



What do patients want from immunotherapy?

Less hype, more realism

- Compared regimens > guidelines
- Integration w/other treatments
- Better care
- “C” word issue (cure)

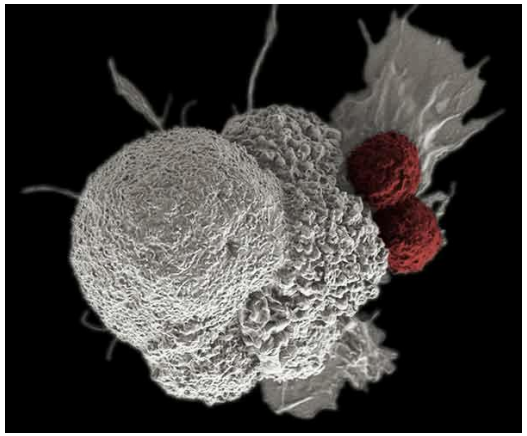
Fewer irAEs

- ≥ grade 2 can be serious
- Autoimmune issues?
- Possible age factors?

Report additional info

- Response rates
  - Comparable to chemotherapy
- Duration of response
- Financial toxicities
- QOL & PROs

CONTEXT



https://www.inspire.com/groups/american-lung-association-lung-cancer-survivors/discussion/opdivo-beware-the-hype-and-commercials/

https://jitc.biomedcentral.com/articles/10.1186/s40425-017-0300-z

http://yourcenter.uvacancercenter.com/autoimmune-disorders-and-cancer-whats-the-connection/

https://www.medscape.com/viewarticle/897946

http://bit.ly/2LD4YPX

Health literacy through the clinical trial process






Recruitment

Effective and useful recruitment materials
- Fliers
- Social media messages
- Website design
- Print and multimedia
- News releases

Consent

Truly informed consent
- Effective, understandable and legal informed consent forms

Retention

Engaging retention materials
- Patient information
- Helpful reminders
- Data collection forms

Results

Health-literate results
- Plain-language clinical trial summaries
- Journal articles
- All media types

Evaluation

Improving all materials and processes via rigorous evaluation
- Formative
- User testing via focus-groups and interviews
- Multimedia

Communication

Collaborative communication between study staff and participants
- Effective, valid, and reliable frameworks for best practice adoption

C3T Clearly Communicating Clinical Trials
Powered by Health Literacy Media

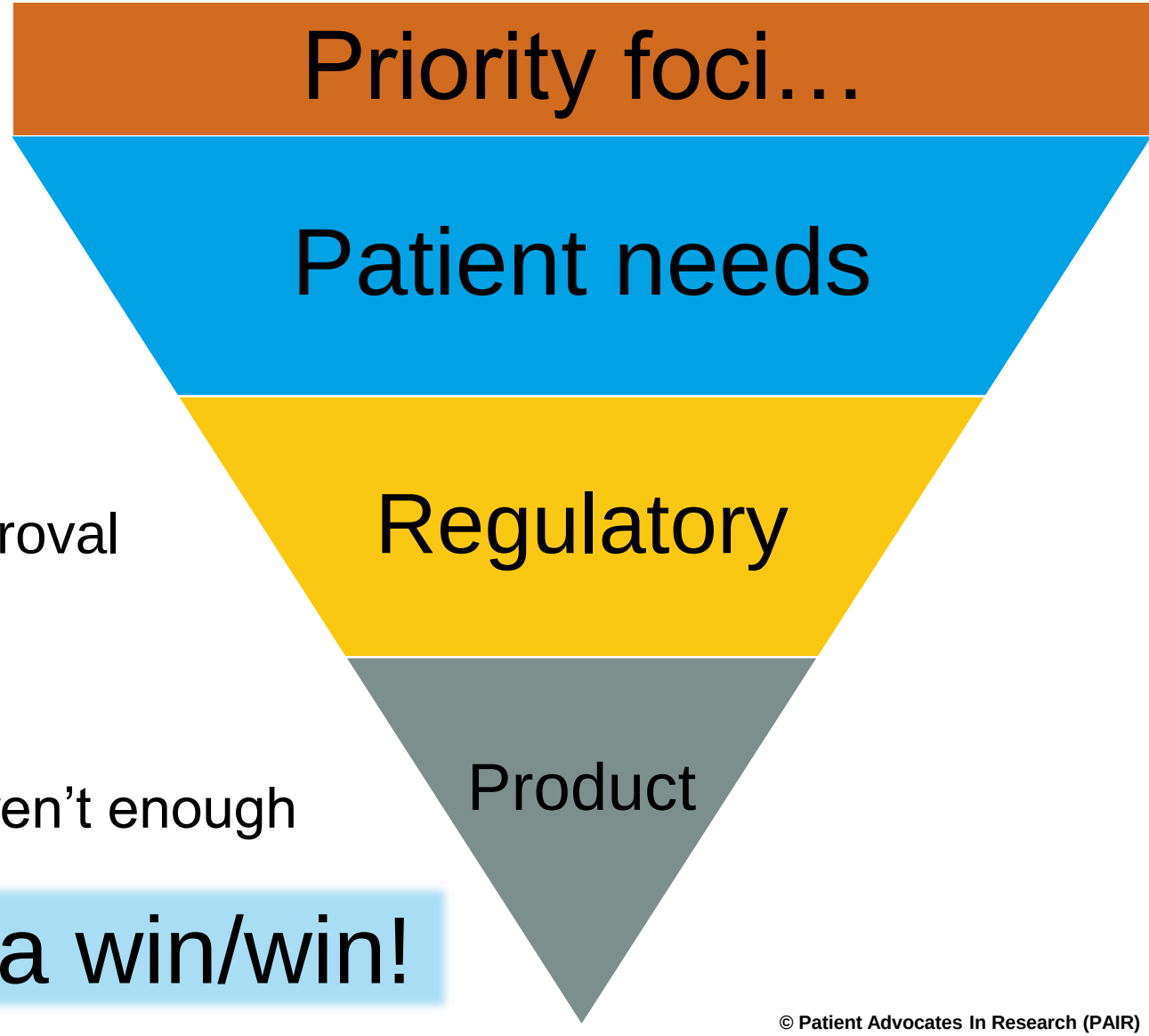
HLM

Time to flip priorities to WIIFP…

What's In It For Patients?
How ***they*** get better results

Before regulatory
Better patient outcomes win approval

Before product
Cool science, delivery & profit aren't enough



Can be a win/win!

Regulators are interested too!

Regulatory Focus™

Regulatory Focus™ > News Articles > 12 > Patient Experience Data: FDA Drafts Guidance

Patient Experience Data: FDA Drafts Guidance

Posted 20 December 2018 | By Zachary Brennan

Thanks to the *21st Century Cures Act*, the US Food and Drug Administration (FDA) on Thursday published new draft guidance to help stakeholders submit a proposed draft guidance on patient experience data.

The 12-page draft guidance, which provides information in a Q&A format, addresses questions relating to both guidance development and other potential pathways for contributing patient experience data.

"Today's guidance document is part of our commitment to advance patient focused drug development and is one of several guidances that we're developing regarding the collection of patient experience data, and the use of such data and related information in drug development," FDA Commissioner Scott Gottlieb said. "This guidance document proposes a roadmap for stakeholders who are interested in developing and submitting proposed draft guidance to the FDA relating to patient experience data."

https://www.raps.org/news-and-articles/news-articles/2018/12/patient-experience-data-fda-drafts-guidance?utm_source=MagnetMail&utm_medium=Email%20&utm_campaign=RF%20Today%20%7C%202%20January

ROI? No, Return On Engagement (ROE)...

Amendments

- Fewer when we're involved in design

Recruitment/Enrollment

- Identify issues, resolutions
- Materials from patient perspective
- Informed consent

Retention/Adherence

- Sanity check in concept/protocol
- ***Endurance*** focus

Net Value =

$35m - $75mm

Vs

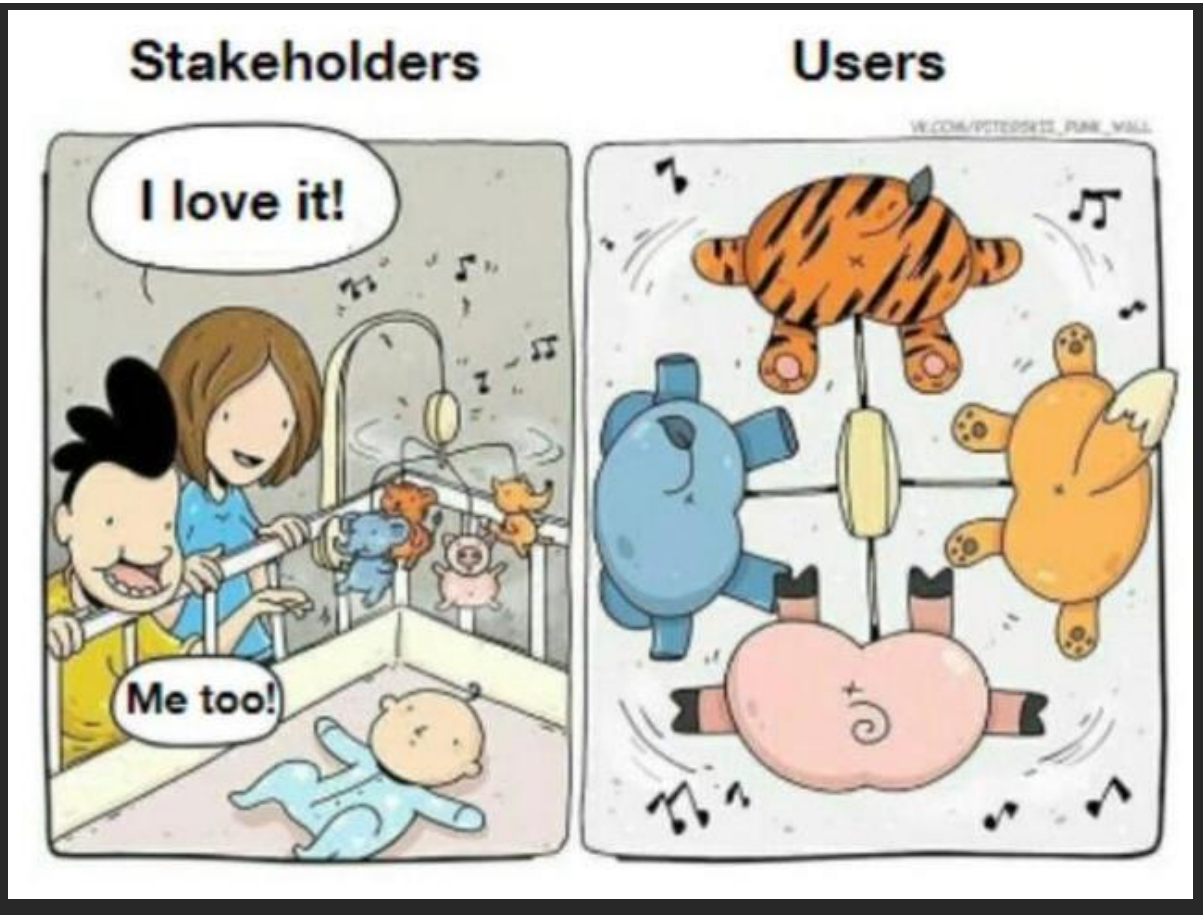
<$250,000 cost

What will **patients** get back?

CTTI Patient Groups & Clinical Trials Project, http://journals.sagepub.com/doi/full/10.1177/2168479017716715

Context…

One final point



Patient Advocates In Research (PAIR)

Where research meets reality

Thank you! Get in touch

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