

Patient community driven alliances

PATIENT ENGAGEMENT IN 2030

How can patient engagement contribute towards building a new ecosystem for drug development & therapy?



Join us!

Thursday, June 9th 2022
Muntgebouw Utrecht, NL
14:00 - 18:30
Registration will be free



admedicum
Business for Patients



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Also: Singularity Bio
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Advisor and former executive several rare disease patient organizations

@CNSDrugHunter

Outline

1. Big picture: patient engagement **in 2030**
2. Patient community driven **alliances** today
3. Patient community driven **alliances** in 2030

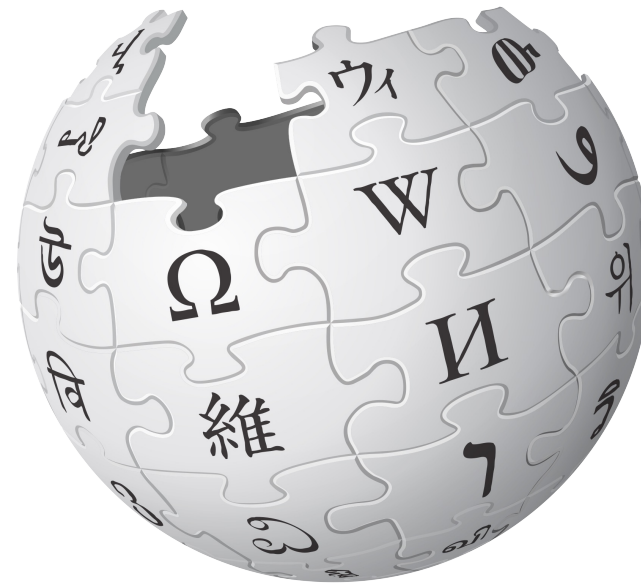
*Personal opinions from my experience in **rare diseases**
and examples from our work at the **Loulou Foundation***



Where patient engagement
will be **in 2030**

Decentralized

knowledge



Bottom-up

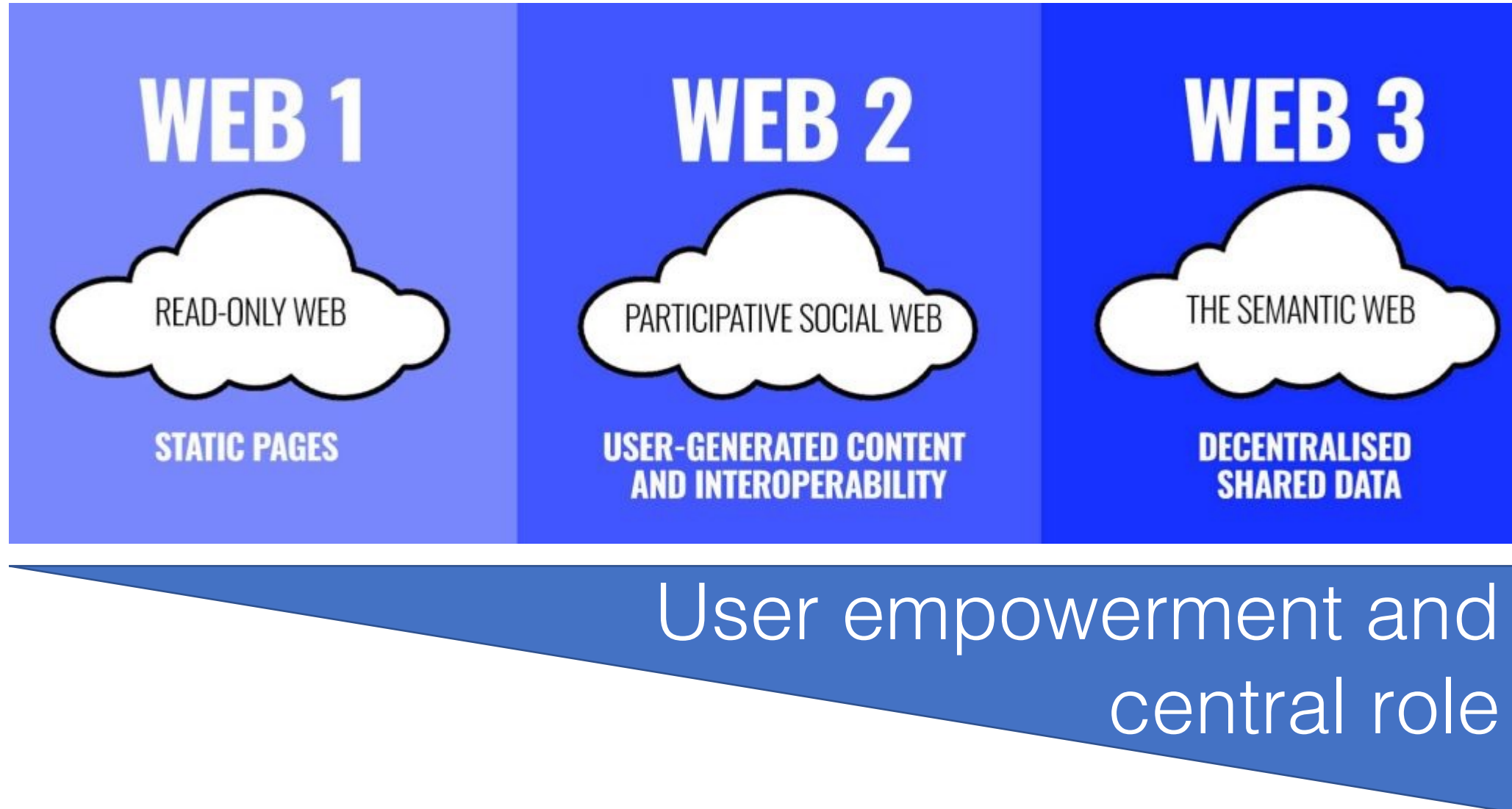
User-generated

High quality

Adaptable

WIKIPEDIA

Decentralized **content**



Decentralized **drug development**

User empowerment and central role

Regulators listen to patients

Patient-generated data and RWE

De-centralized clinical trials

Patient group leadership

Patient input from very early stages

N-of-1 trials (skip pharma)



Patient community driven
alliances today

Change in mindset to **build with patients** already widespread

But execution still **lead** from the patient community

In today's world you **cannot build without** the user, and the patient community is a superb partner



**FDA and EMA engagement
on benefit-risk and endpoints**



cdkl5registry.org

**Patient registries and
outcome measures**

Prepare community

Regulatory education

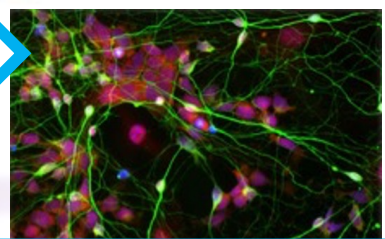
Clinical development

Preclinical research

Discovery research



**Funding, network,
knowledge**



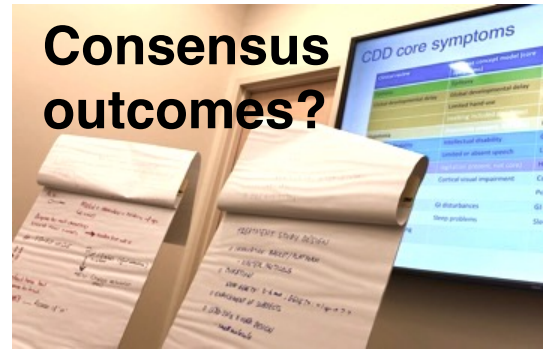
Open-access tools. Fund key experiments.



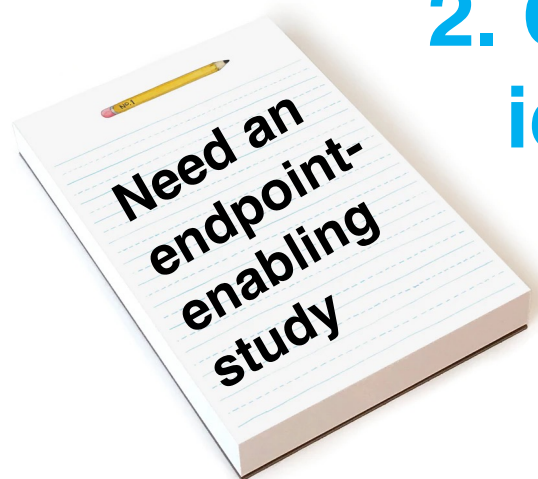
Case study:

The backstage of a massive
patient community-driven **alliance**

1. Reasonable question



2. Crazy idea



3. Mental barrier



5. Logistic wall



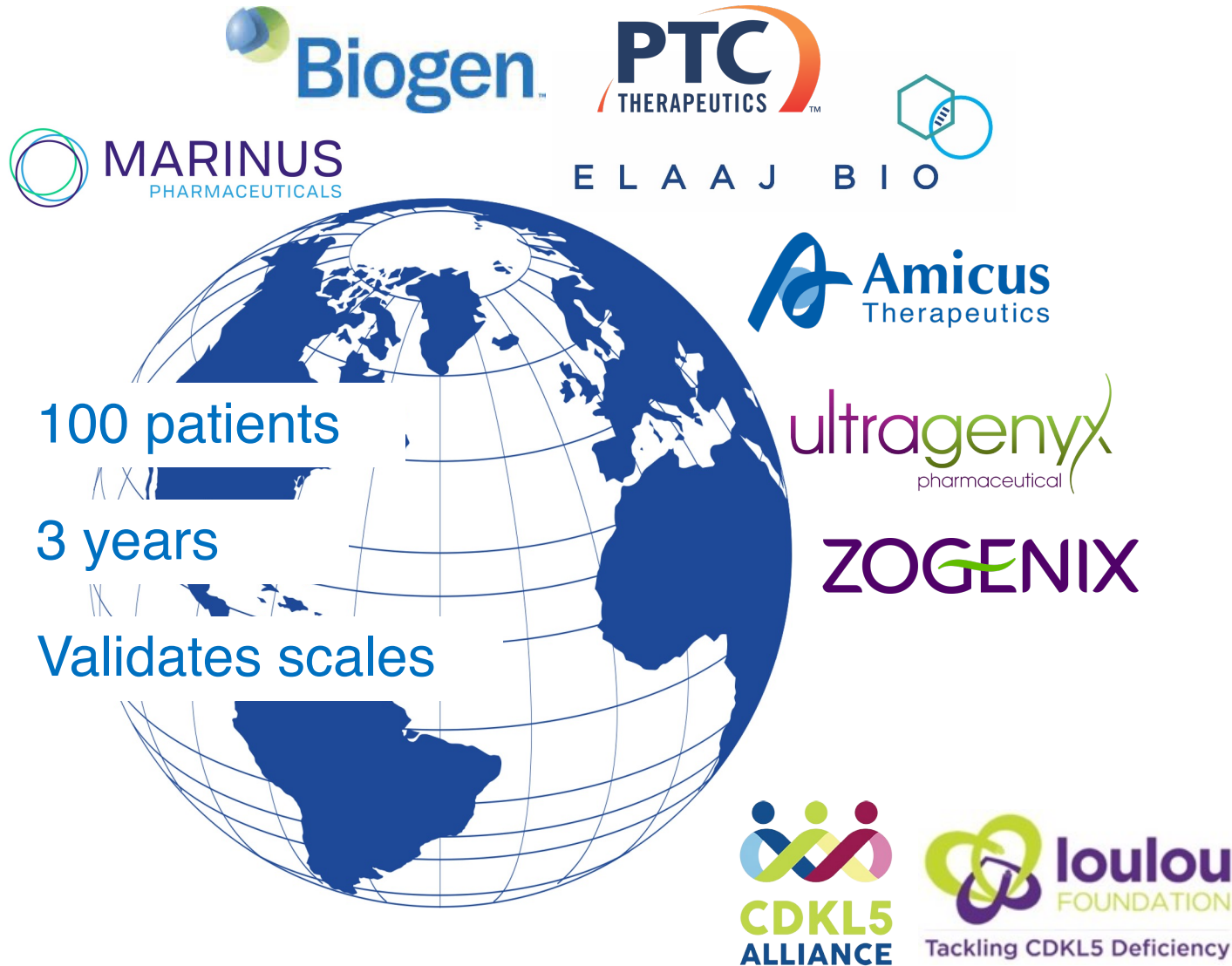
**Sign up CRO
Hire ClinOps + ClinDev
Lawyers!**

6. Re-define precompetitive

4. Open mind



FDA endorses design and partnership



100 patients

3 years

Validates scales

7 companies

Enable trial design

Minimize placebo

Global trial network

By re-defining what is **pre-competitive**
we created much **value** for patients
and companies, and solved the
funding for the study

We are the **Proof of Concept** for
community-driven alliances

Now this needs to **scale**

Patient community driven
alliances tomorrow

By **2030...** the patient community has become **professionalized** involving experts beyond patients



Normal **career path** for professionals



Transition from charity to **essential party**



Enabled by project **funding from industry**

By 2030... an ecosystem of “bilingual” organizations supports plug-and-play patient alliances and operations

Examples of organizations supporting de-centralized drug discovery, development and partnering:

Registry/studies:



Preclinical & strategy:



Engagement pharma-patient:



By **2030**... a **social contract** makes biopharma companies help build knowledge and de-risk fields



Participating and co-funding community efforts like research tools and registries is **the ticket to pay** to be able to develop a treatment in that space

By 2030... a social contract makes bio-pharma companies help build knowledge and de-risk fields



KIF1A.ORG
@KIF1A

#ThisIsKIF1A from @kat_atchley: “Imagine if the first 100 people diagnosed with COVID were told, ‘Wow, that’s unfortunate. Why don’t you come up with the money and convince enough scientists and pharmaceutical companies to study this unknown disease with no treatment? ...”

6:08 PM · Jun 13, 2021 · Twitter Web App

4 Retweets 1 Quote Tweet 9 Likes



KIF1A.ORG
@KIF1A

Make sure you work fast though, because millions of other lives now and in the future are counting on you.’

That’s what it’s like for KIF1A and rare disease families around the world. #RareDiseaseTruth ...

6:08 PM · Jun 13, 2021 · Twitter Web App

2 Retweets 4 Likes

By **2030**...

Patient at the **center** of drug development, supported by

professionalization of the patient community,

an **ecosystem** of bilingual organizations,

and a **social contract**

