
citizen
health®

Empowering patients, advocates, researchers, and drug developers with **real-world insights** from **patient-consented data**

How it works

1

Build high-density patient communities

by lowering the burden on patients to participate and engage

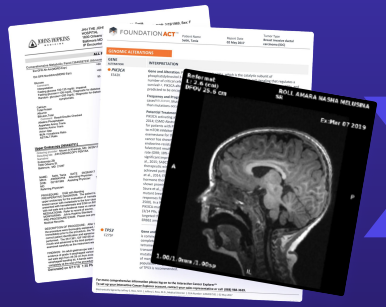
70%

organic community growth

2

Collect complete & compliant data

with a proprietary and automated regulatory compliance framework



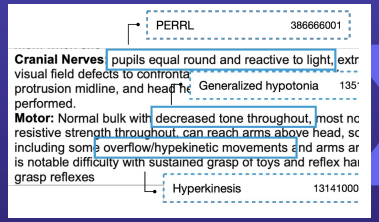
14k+

healthcare providers

3

Transform records into regulatory-grade data

at a richness, speed & scale that was not previously thought possible



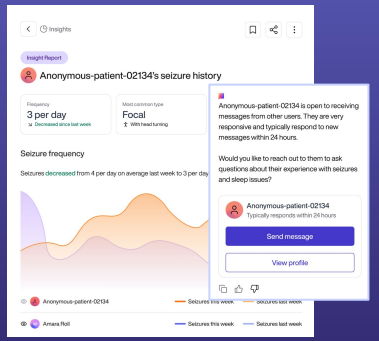
460+

data points per patient

4

Deliver timely & actionable AI insights

illuminating new discoveries for pharma, patients, and advocates



10+

years data on each patient


Every day of delay to market
costs **$600K-$8M per drug**

For biotech & pharma sponsors

We streamline process **from trial design, recruitment to LTFU and label expansion**

Without Citizen

12-30+ years

Discovery/Preclinical	Pre-IND	Phase I & II	Phase III	Approval	LTFU/Post Mktg
5-10 years		2-5 years	3-5 years	1-2 years	1-10+ years

Citizen-Powered

7-15+ years

Discovery...	Pre-IND	Phase I & II	*Accelerated Approval*	LTFU...
3-7 years		2-4 years	1-2 years	1-10+ years

Citizen can shave up to 40% off a program, resulting in tens of millions in savings

Not your typical RWD vendor.

Informed and empowered, **our patient communities** consent to share complete medical histories to advance better treatments

Regulatory-grade, longitudinal data

Submission-ready datasets, accepted by FDA to replace traditional in-person study requirements (e.g., Praxis IND)

Comprehensive patient medical history

Unifies unstructured medical records, genetics and imaging across systems, from birth / diagnosis

High-density patient communities

Built with 60+ long-term advocacy partners - enabling trusted, aligned engagement

Recontactable, engaged patients

Trusted relationships enable recontact for faster recruitment and effective long-term follow up

Traditional NHS

- ❌ 3-5 years
- ❌ Burdensome in-person process
- ❌ Patient burden leads to data gaps
- ❌ Limited data points captured

Citizen powered

- ✅ 6 months
- ✅ No patient burden
- ✅ 10+ years of FDA-grade longitudinal data
- ✅ Comprehensive data (463 data points/patient)

Spotlight

Praxis obtained **IND clearance from FDA 4 years early** with Citizen Data

Citizen delivered full natural history data **in 6 months—fully digital, no site visits**—enabling the first-ever **FDA-approved use of RWD to replace in-person NHS** in rare disease.

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Problem

When good science meets **bad design**

90%
Drug candidates fail in trials

40-60%
Fail due to flawed trial design

- Suboptimal endpoints
- Poorly prioritized biomarkers
- Overly restrictive I/E criteria
- Unclear unmet needs

Citizen helps you **find the right patients, endpoints and biomarkers** from the start

Case study

Rethinking the endpoint to **save the trial**

Challenge

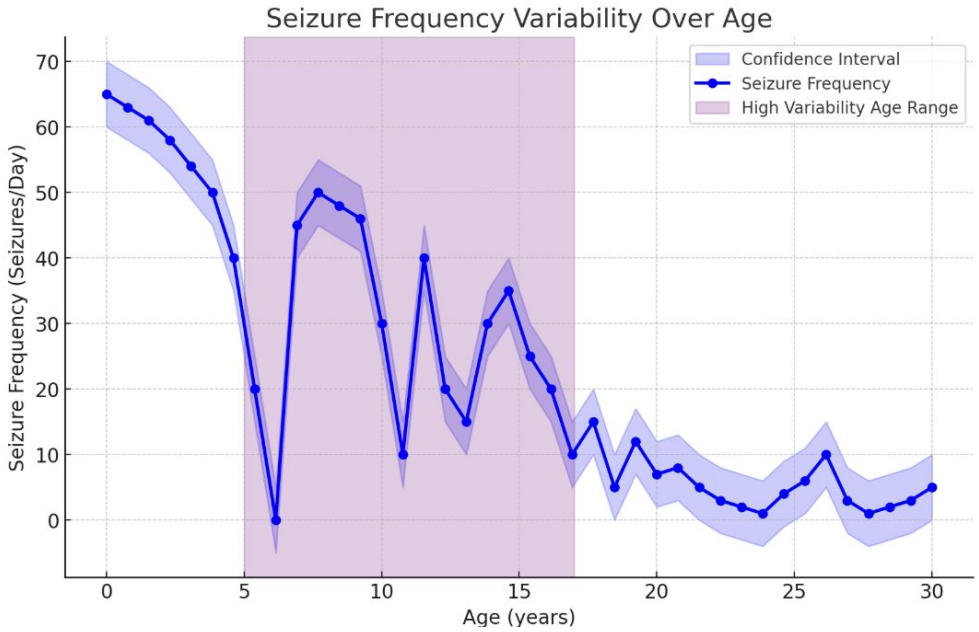
Seizure frequency is often used as a key clinical endpoint **across age groups**

What we found

Patient data showed **significant variation by age** making seizure frequency an **unreliable endpoint**

Our recommendation

Prioritize ages 17+ in inclusion criteria for **clearer efficacy signal** and **smoother path to approval**



Case study

Greater relevance. Better outcomes.

Challenge

Narrow RCT criteria excludes patients with diverse seizure profiles and age ranges **limiting generalizability**

What we found

We revealed critical differences between RCT and real-world cohorts. Without these insights, key patients would have been excluded.

Our recommendation

Broaden I/E criteria to reflect real-world profiles - wider age ranges, seizure profile and treatments

Comparison of Patient Characteristics: RCT vs Real-World

Variable	RCT (N=40)	Real-World (N=60)	P-Value
Age (years), Mean ± SD	6.1 ± 1.5	7.1 ± 1.9	**0.005**
Seizure Frequency (per month), Mean ± SD	75.9 ± 19.5	106.0 ± 31.0	**<0.001**
Duration of Epilepsy (years), Mean ± SD	3.3 ± 0.9	4.3 ± 1.6	**0.001**
Sex (Male %)	25 (62.5%)	30 (50.0%)	0.305
Developmental Delay (Yes %)	34 (85.0%)	57 (95.0%)	0.175
Mutation Type (Missense %)	20 (50.0%)	30 (50.0%)	0.557
Drug X (Yes %)	18 (45.0%)	32 (53.3%)	0.452
Drug Y (Yes %)	5 (12.5%)	38 (63.3%)	**<0.001**

Note: Pink-highlighted rows indicate statistically significant differences ($p<0.05$)

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Case study

Signal over noise: Biomarkers that guide trials

Challenge

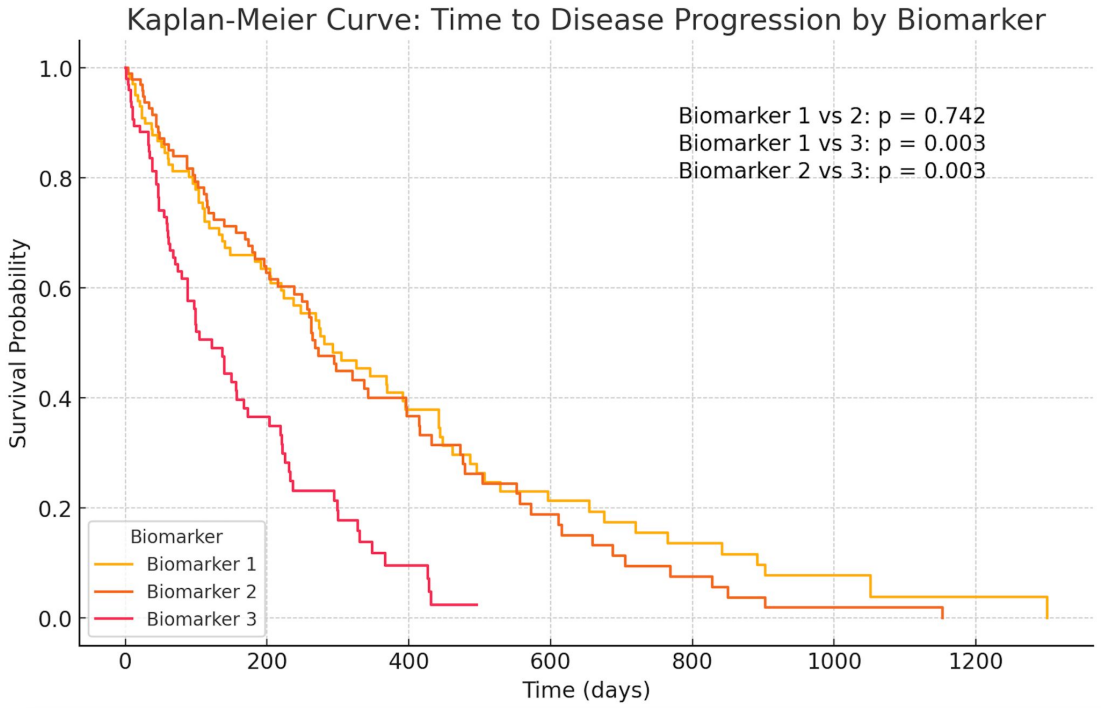
There were **no reliable biomarkers** to guide early-phase patient selection

What we found

We identified a predictive biomarker linked to faster progression (Biomarker 3)

Our recommendation

Prioritize high-need patients leveraging Biomarker 3 in Phase 1/2



*The p-values are reported from log-rank tests

Case study

Proving the Need. Powering regulatory filing.

Challenge

Gaps in disease knowledge and unmet patient needs complicate early-stage development

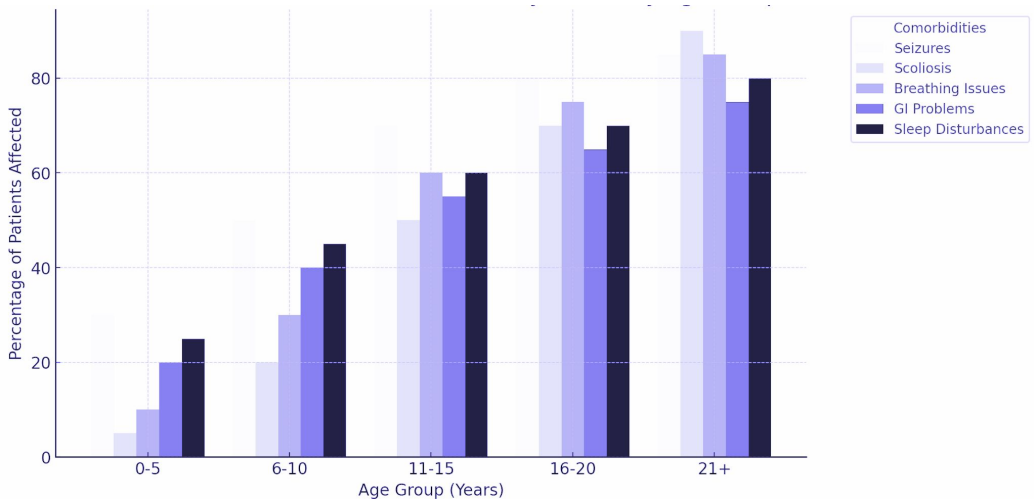
What we found

We highlighted increasing comorbidity burden by age across all comorbidities

Our recommendation

Leverage real-world comorbidity data to **articulate unmet needs in NDA filings**

Rate of the most common comorbidities across age groups



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Problem

Hidden roadblocks in rare disease trials

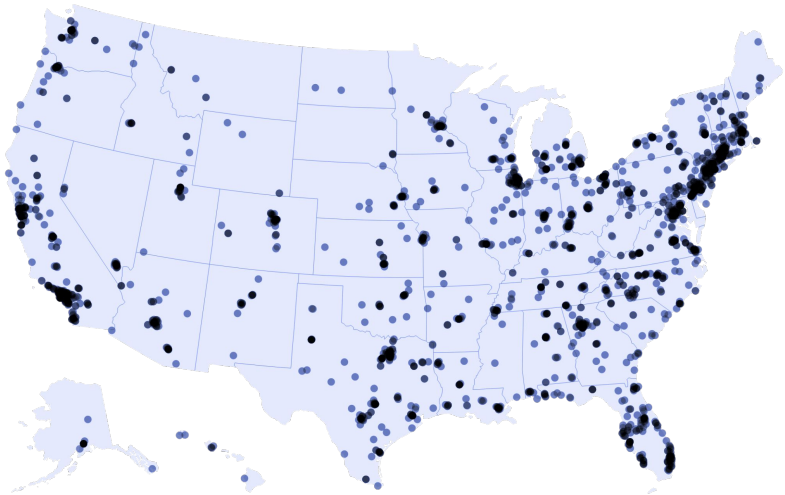
80%
Trials fail to meet enrollment timelines

11%
Sites fail to enroll a single patient

- Hard to find patients matching I/E
- Poor patient engagement
- Randomized trials are impractical

Case study

From guesswork to **precision mapping I/E eligible patients**



Challenge

Unclear where to find patients matching exact I/E criteria (e.g., females with MECP2 mutation, age 2-18)

Solution

We examined **geographical distribution of patients matching I/E** and **identified patient clusters at key institutions** across the country

Case study

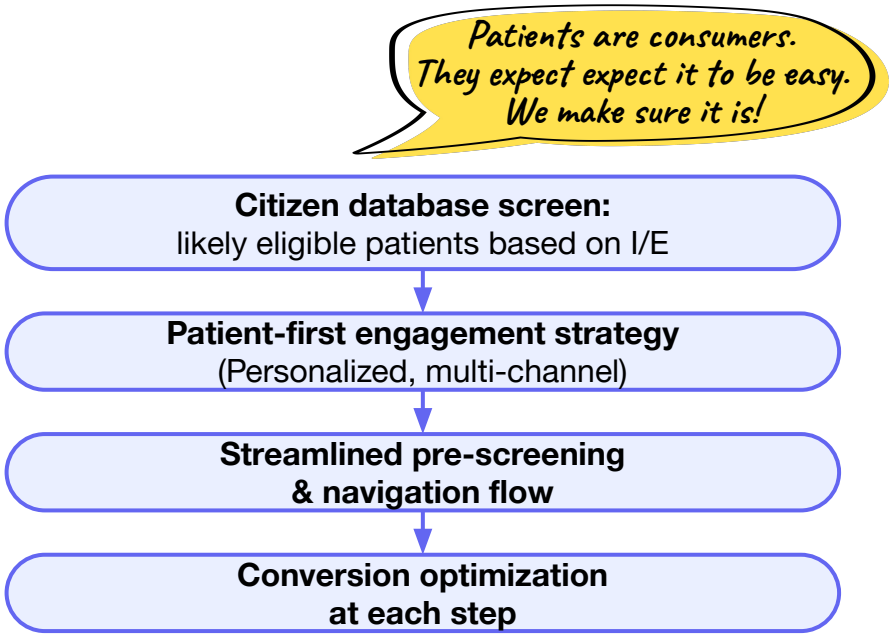
A consumer-focused, personalized approach to patient enrollment and engagement

Challenge

Lack of effective **patient engagement strategy** can cause **delays in enrollment timeline** or **force trial termination**

Solution

We **engage patients where they are**, and guide them through enrollment with a **streamlined, patient-friendly flow**



Case study

Credible control arms with clinical-grade data

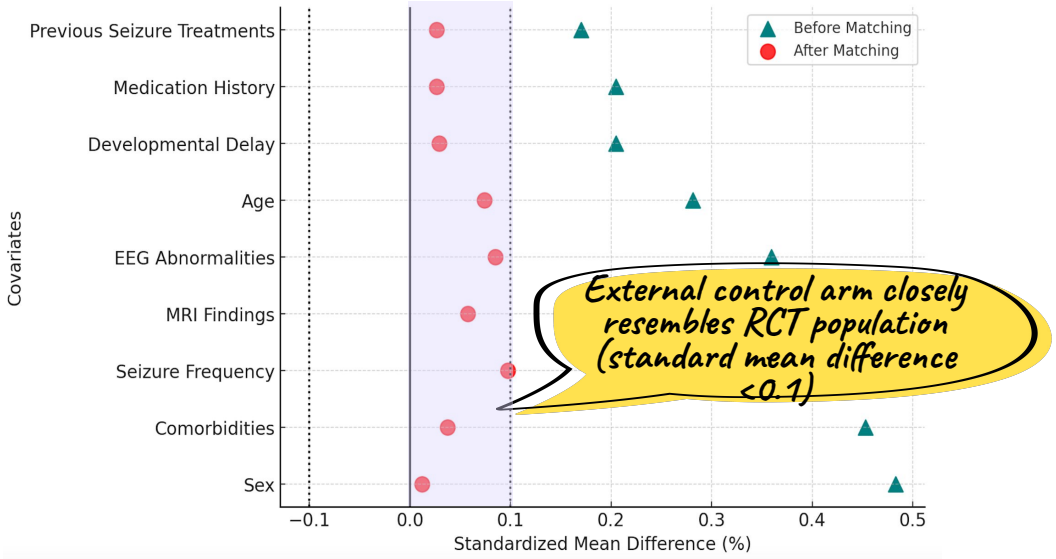
Challenge

In rare disease trials, **traditional double-blinded studies are often not optimal — or ethically impossible**

Solution

Provided statistically matched external control arms built from real-world data, helping sponsors **generate credible evidence without needing a placebo group**

Comparability between RCT population and external control arm



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Problem

Approval is not the finish line

Up to 15 Years
Long-term follow-up required for gene therapy

11%
Dropout rate with logistics/travel as the top driver

- ⊗ **No credible LTFU plan**
- ⊗ **Patient burden and dropout**
- ⊗ **Label expansion too costly**
- ⊗ **Rising demand for proof**
  (Efficacy, safety and value)

Case study

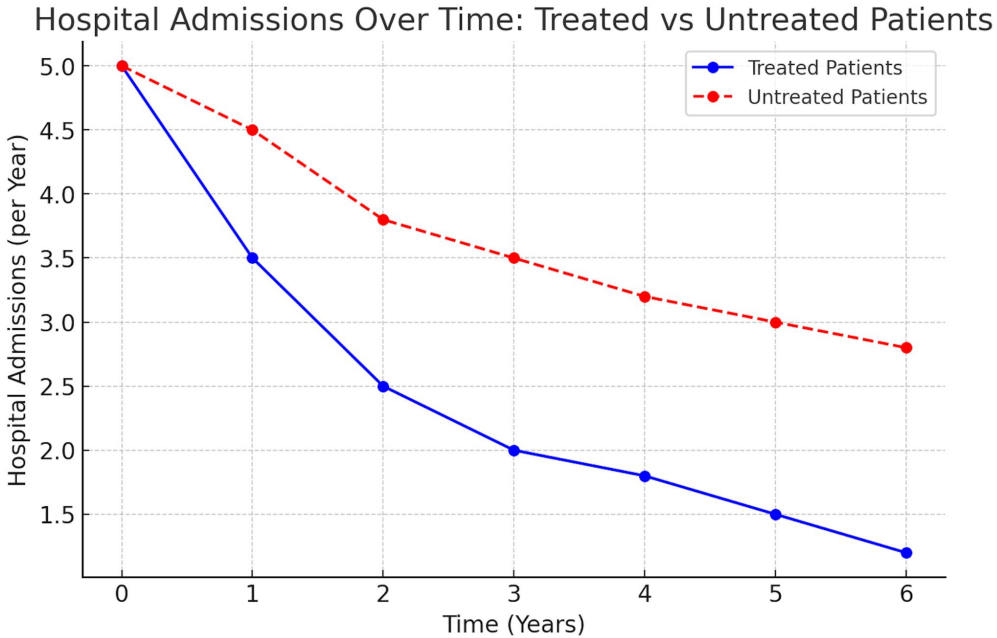
Approval demands a LTFU plan: **Prospective follow up without patient burden**

Challenge

Regulatory agency demand **long-term follow up (LTFU)** of effectiveness of treatment A among patients treated for X condition. However, **patient burden and dropout** could hurt credibility with regulators

Solution

Inclusion of Citizen Health into trial protocol **enables prospective follow-up of patients**. Demonstrated reduced hospital admissions among treated patients



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Case study

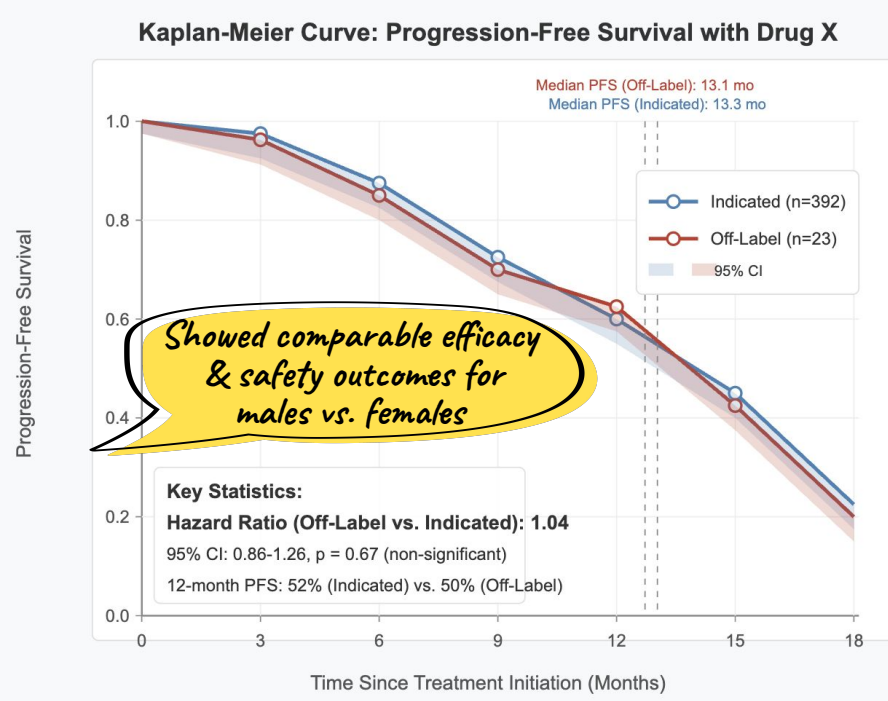
Subpopulation label expansion without needing a new trial

Challenge

Clinical trials are costly! And can be **difficult to develop for sub-populations with rare diseases** given small sample size

Solution

Leveraged RWD to support label expansion from female-only to include males



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Case study

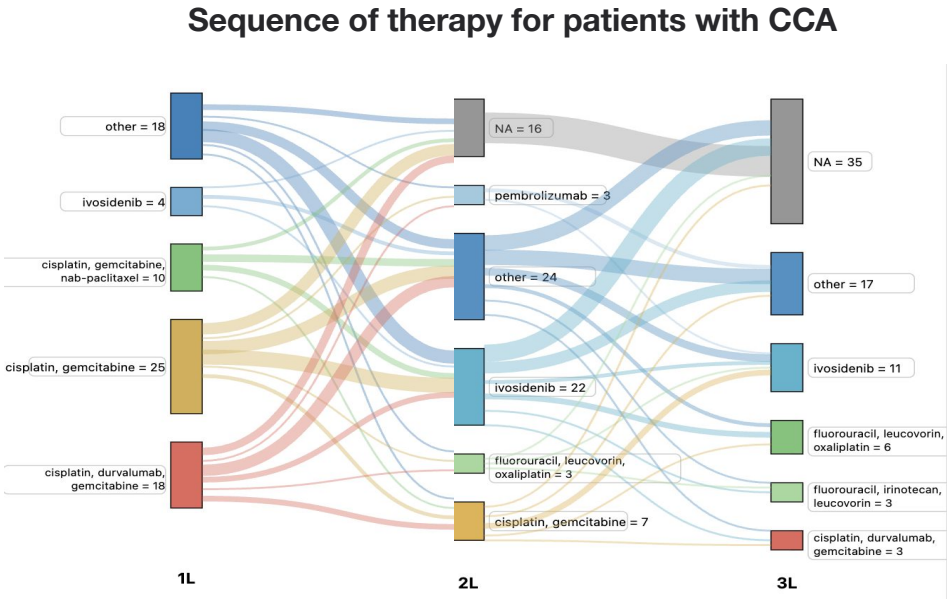
From label to reality: Discover your therapy's real role in care

Challenge

Uncertainty around treatment pattern and how Drug X was being used in real-world clinical practice — especially outside its indicated setting

Solution

Revealed that Drug X was prescribed earlier than labeled, was well-tolerated, and supported its repositioning for **updated clinical guidelines**



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