Barriers to Clinical Translation

Impossibly rare cancer
- Never studied prospectively
- Perceived to be a pediatric malignancy

JQ1 as a chemical probe
- Not optimized for DMPK properties
- Immediate opportunities for translation?

Drug development funding/infrastructure
- Non-traditional academic research
- Sense of responsibility and immediacy
The International NUT Midline Carcinoma Registry Team

Top from left:

Bottom from left:
Daniel Bauer, Chris Fenske, Kelly Straight.

Families » Doctors » Enroll »
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Cell (2011)

Nature (2011)

PNAS (2011)
target selection irrespective of perceived druggability • facilities appropriate for discovery research • immediate use of emerging animal models • collaborative support of our patients • flexibility in licensing for further clinical development • access to a massive research infrastructure • research motivated by creative impact insights derived from clinical experience • lack of profit motive a sense of urgency and immediacy • broad dissemination of chemical probes • ability to crowd-source new indications • long timelines • toward an open-source model of drug discovery increased velocity of knowledge around a new chemical probe generation of relatively inexpensive assets • flexible development of clinical candidates • a tradition of creativity and research excellence • peer-review of research at every stage • support of institutional leadership at the dana-farber cancer institute maximum return of revenue to research and development • no bureaucracy • a culture of collaboration and scholarship generation of inexpensive assets • publication of chemical probes more relaxed legal restrictions on sharing and discussing research
“…I will respect the hard-won scientific gains of those physicians in whose steps I walk, and gladly share such knowledge as is mine with those who are to follow…”

Hippocrates

**Decentralize** therapeutic discovery

**Declassify** drug discovery and development data

**Empower** those who succeed to succeed again

**Accelerate** therapies through to human clinical investigation