FREE TO CHOOSE MEDICINE

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GOAL: BETTER DRUGS SOONER AT LOWER COST

Antiquated and obsolete FDA regulatory process

- Bureaucratic and slow-moving organization
- Paranoid about potential negative publicity
- Insulated from competitive pressure

STATUS QUO

- Relentless demand for more extensive clinical testing
- Sky-high prescription drug prices
- Access delayed… invisible graveyard of those denied opportunity for innovative new drugs











BETTER DRUGS SOONER AT LOWER COST

THE WORLD HAS CHANGED

- Accelerating medical knowledge
- Personalized medicine
- Internet
- Patients' enthusiasm to share data
- Big data analytics

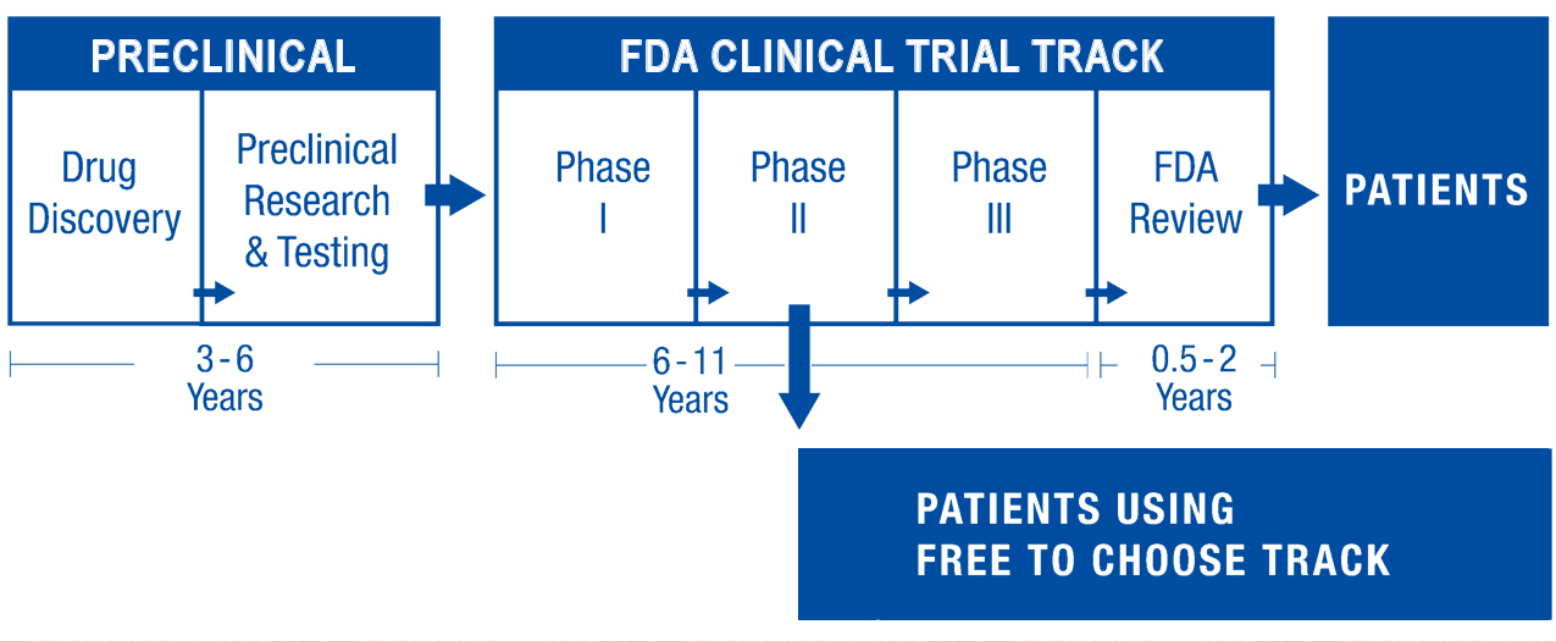
FREE TO CHOOSE MEDICINE

Consumer choice an competition

Informed decisions about life-changing new drugs

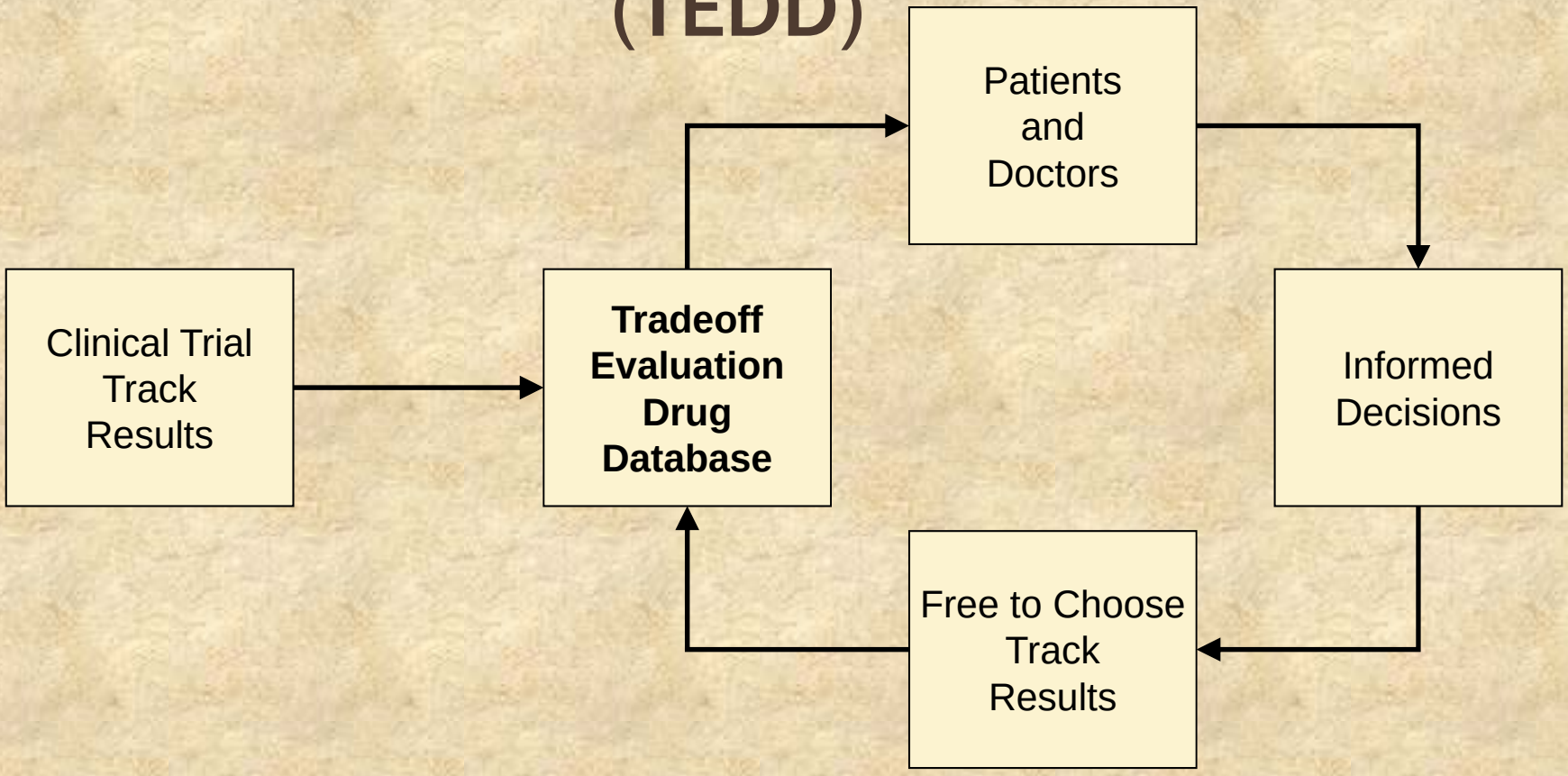
Three components

#1 FREE TO CHOOSE TRACK OPERATED INDEPENDENTLY OF THE FDA



FTCM legislation would create a dual track system that preserves the existing FDA clinical trial process while offering patients an alternative. Patients, advised by their doctors, would be able to contract with a drug developer to use not-yet-approved drugs after Phase I safety trials are successfully completed and one or more Phase II trials have demonstrated continued safety and initial efficacy. The resulting early access could make FTCM drugs available up to seven years before conventional FDA approval, which entails Phase III randomized control trials and a lengthy FDA review before the FDA makes an approval decision.

#2 TRADEOFF EVALUATION DRUG DATABASE (TEDD)

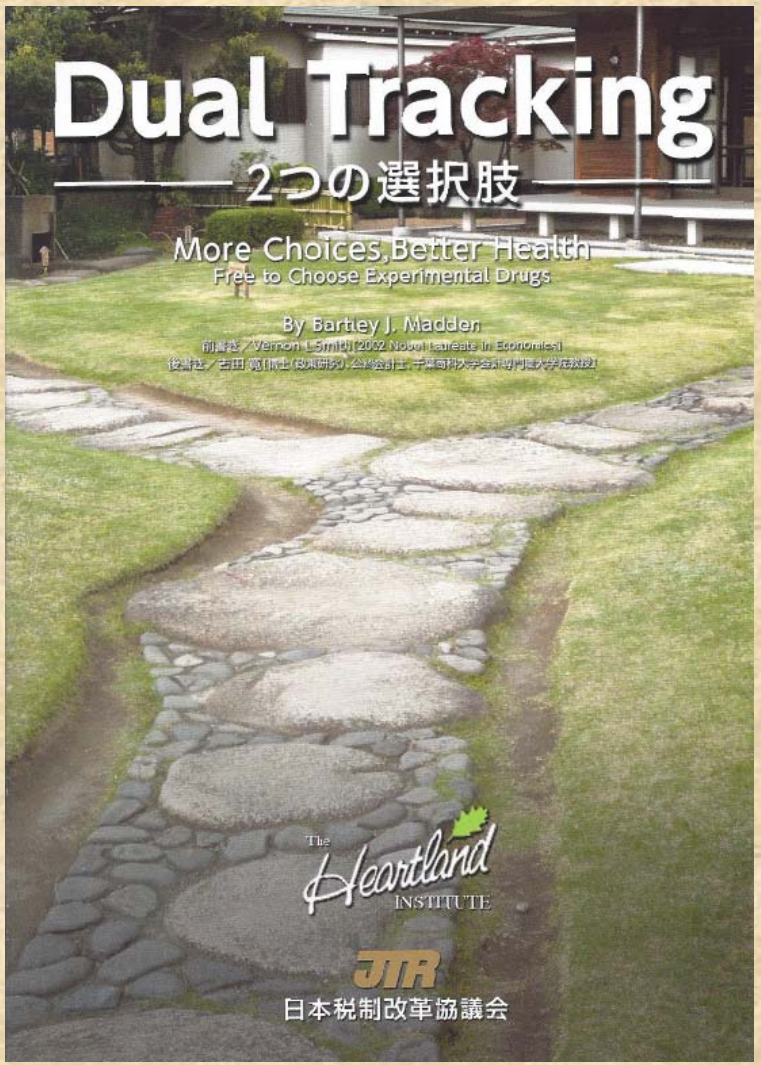


TEDD makes available to the public through a government-sponsored web portal the information that patients and doctors need to make informed decisions about a FTCM drug's potential benefits and risks before choosing to use it. TEDD would be a treasure trove of data about patients' genetic makeup, biomarkers, and treatment results. Data from a heterogeneous patient population that mimics real-world use better than tightly controlled clinical trials do. The FTCM track and TEDD constitute a self-adjusting system wherein increased usage of FTCM drugs corresponds to demonstrated effectiveness and vice versa.

#3 OBSERVATIONAL APPROVAL

- FDA could grant Observational Approval due to compelling **safety and effectiveness demonstrated in use**.
- Based not on randomized control trial data, but on large-sample, treatment data for **real-world patients**.
- Consistent with recent regulatory advancements in **Japan**.
- Drug developers would be motivated to charge **lower prices** in order to gain increased usage for their FTCM drug.
- TEDD's **up-to-date observational data** would guide patients and doctors, especially when genetic/biomarker tags identify patients who experienced especially favorable treatment outcomes.
- Observational Approval would **expedite insurance reimbursement**.

FREE TO CHOOSE MEDICINE IN JAPAN



This 2007 Japanese translation of an early FTCM booklet was heavily promoted to Japanese politicians by Masaru Uchiyama (Mr. You), President of the Japanese for Tax Reform.

FTCM principles contributed to the passage in 2013 of Japanese legislation permitting early access to regenerative medicine drugs which will be monitored via observational data.

PREFERRED 21ST CENTURY BUSINESS AND REGULATORY MODEL

"Unless fundamental change takes place, biopharmaceutical companies may well have to deal with **government price controls** which will have a disastrous impact on long-term innovation. A market-based solution—Free To Choose Medicine (FTCM)—has been widely published and implemented in Japan. … With favorable FTCM experience, expect the country to expand freedom of choice to cover a great many diseases. We can look to Japan to see a test of the preferred 21st century business and regulatory model for the biopharmaceutical industry."

Bartley J. Madden and Vernon L. Smith, 2015, "Give the FDA Some Competition With Free To Choose Medicine," *Forbes Online.*

SUMMARY

- Right To Try legislation for terminally-ill patients has passed in 32 states. Although it does not address structural FDA reforms like FTCM, it is a huge success in demonstrating **popular support for freedom of choice.**

- With the FTCM paradigm, everyone learns at a rapid pace. FTCM is a **dynamic, self-adjusting system** where we learn about results and subpopulations of patients who do extremely well or poorly and we are able to make informed decisions.

- Past FDA reform legislation focused on incremental improvements and invariably assumed that the FDA must play the central role. **Who has ever seen a government agency willingly reduce its own power? The FTCM track and the TEDD must operate independently of the FDA** to bring private-sector innovation and efficiency to the slow-moving bureaucratic FDA process.

SUMMARY

- FTCM would bring **competition** to the FDA by enabling the public to evaluate how patients fare with freedom of choice.
- Expect **lower drug prices** because streamlined clinical testing and review translates into greatly reduced costs. This enables drug developers to charge much less while maintaining their profitability. Keep in mind that drug developers would face heightened **competition** due to the rapid introduction of new, FTCM drugs.
- FTCM puts a **premium on scientific skill** in developing breakthrough medical treatments and efficiency in commercializing new innovations.
- Every American should have the **right** to make their own informed decisions that can improve and save lives.
- We are working to have a **FTCM bill** introduced in Congress and hopefully supported by the Trump administration.