Right to Trial & FDA Upgrade Act

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Every patient gets the right to try. Every treatment teaches us something.

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

1 TITLE I: PURPOSE AND DEFINITIONS

1.1 SEC. 101. SHORT TITLE

This Act may be cited as the **"Right to Trial and FDA Upgrade Act."**

1.2 SEC. 102. PURPOSE

The purpose of this Act is to establish a universal right to seek investigational treatment after initial human safety testing while protecting voluntary supply, informed consent, reasonable safety standards, and the production of useful evidence.

1.3 SEC. 103. FINDINGS

Congress finds the following:

1. Existing federal expanded-access and right-to-try pathways leave many patients and conditions outside their practical reach.
2. Access may help the patient receiving a treatment. Standardized outcome collection can also help every later patient facing the same decision.
3. Permitting willing suppliers to earn revenue after initial human safety testing can finance further investigation of treatments that could not otherwise pay for phase 2 or phase 3 trials.
4. Pragmatic and decentralized trials can study treatments in routine care and include patients who would not qualify for conventional trials.
5. Federal law should protect access without promising that a treatment works, requiring anyone to supply it, or requiring anyone else to pay for it.

1.4 SEC. 104. DEFINITIONS

In this Act:

1. **Eligible investigational treatment** means a drug, biological product, device, diagnostic, procedure, behavioral intervention, or combination of them that:
   (A) has completed a phase 1 clinical investigation or has a comparable human safety record documented by a licensed medical facility or recognized research institution and found sufficient by the qualified provider and reviewing ethics board to characterize initial safety and dosing for the treatment and route of administration;
   (B) is not subject to an active clinical hold applicable to the proposed use;
   (C) is manufactured, stored, prescribed, and administered under applicable quality and safety standards; and
   (D) is supplied by a person legally entitled and willing to manufacture or provide it.
2. **Condition** includes a disease, injury, disability, symptom, functional impairment, or biological process associated with aging.
3. **Qualified provider** means a licensed health professional acting within the professional's lawful scope of practice.
4. **Right-to-trial protocol** means a prospectively registered plan for providing an eligible investigational treatment and collecting the minimum evidence required by section 203.
5. **Qualified evidence system** means a public, nonprofit, or private registry or platform that satisfies section 301.
6. **Secretary** means the Secretary of Health and Human Services, acting through the Commissioner of Food and Drugs where appropriate.

2 TITLE II: UNIVERSAL RIGHT TO TRY AND LEARN

2.1 SEC. 201. RIGHT TO ACCESS

(a) **In General.** A patient may seek, purchase, and use an eligible investigational treatment for any condition under a right-to-trial protocol when a qualified provider recommends the treatment and a willing supplier agrees to provide it.

(b) **Independent Federal Pathway.** Compliance with this Act is an independent federal pathway and does not require an investigational new drug application, investigational device exemption, individual expanded-access authorization, premarket approval, clearance, or licensure. Solely for treatment provided under this Act:

1. a drug or biological product is exempt from sections 502(f), 503(b)(4), 505(a), and 505(i) of the Federal Food, Drug, and Cosmetic Act, section 351(a) of the Public Health Service Act, and parts 50, 56, and 312 of title 21, Code of Federal Regulations, except that the labeling requirements in section 312.6 continue to apply;
2. a device or diagnostic is exempt from sections 501(f)(1)(B), 502(f), 502(o), 510(k), 513(f)(2), 515, and 520(g) of the Federal Food, Drug, and Cosmetic Act and parts 50, 56, and 812 of title 21, Code of Federal Regulations, except that the labeling requirements in section 812.5 continue to apply; and
3. the restrictions on prior charging authorization, cost recovery, promotion, and commercial distribution in sections 312.7, 312.8, and 812.7 of title 21, Code of Federal Regulations, do not prohibit truthful promotion, commercial distribution, or the price permitted by section 205.

(c) **Protections Retained.** Nothing in subsection (b) exempts a person from applicable manufacturing and quality standards, prohibitions on false or misleading statements, or the consent, ethical review, evidence, safety-reporting, and liability requirements of this Act. No person may represent an eligible investigational treatment as safe or effective beyond what reliable evidence supports.

(d) **No Compelled Participation.** Except for the duty under section 203(g) to record the disposition of a formal request received through a qualified evidence system, nothing in this Act requires a manufacturer, provider, facility, ethics board, evidence system, insurer, employer, or other person to manufacture, recommend, provide, administer, review, record, or pay for a treatment.

(e) **Clinical Judgment.** A provider may decline or discontinue treatment when the provider reasonably believes that the expected risks exceed the expected benefits for the patient.

2.2 SEC. 202. PATIENT ELIGIBILITY AND INFORMED CONSENT

(a) **Eligibility.** A patient is eligible when:

1. a qualified provider has documented the patient's condition and recommendation;
2. the patient has considered approved treatments reasonably available for the condition; and
3. the patient or a legally authorized representative has given informed consent.

(b) **Required Disclosure.** In plain language, the consent must:

1. identify the treatment and state that it is investigational and may never be shown safe or effective;
2. identify reasonably available approved alternatives;

3. describe known and reasonably foreseeable risks, possible benefits, and the best, worst, and most likely outcomes;
4. explain what remains unknown;
5. state that participation is voluntary and may be ended at any time;
6. state whether an insurer or public program is expected to pay;
7. disclose the total price, included services, payment timing, refund terms, and reasonably foreseeable additional costs; and
8. explain required data collection, permitted uses, privacy protections, and public reporting.

(c) **Form.** Consent may be completed on paper or through a digital process that permits questions and records the patient's agreement. The Secretary may publish model language but may not require a particular technology.

2.3 SEC. 203. EVERY TREATMENT USE GENERATES EVIDENCE

(a) **Prospective Registration.** Before treatment begins, the right-to-trial protocol must be registered with a qualified evidence system and identify the treatment, condition, eligibility criteria, outcomes, follow-up period, safety-monitoring plan, and the supplier, sponsor, provider, or evidence-system operator responsible for evidence submission.

(b) **Minimum Evidence.** Subject to subsection (c), the responsible party must collect and submit the following information for every treatment use:

1. baseline condition and relevant patient characteristics;
2. treatment, dose, duration, and important concurrent treatments;
3. clinical and patient-reported outcomes;
4. discontinuations and serious adverse events; and
5. the completeness and timing of follow-up.

(c) **Follow-Up Completeness.** Each protocol must prespecify a minimum follow-up schedule and completion target and must obtain at least one post-treatment outcome record unless the patient withdraws, dies before the scheduled assessment, or cannot be reached after the protocol's required attempts. For each exception, the responsible party must submit the reason, the dates and methods of attempted contact, and the patient's last known clinical status. The evidence system shall publish protocol-level completion rates, and the reviewing board may require a corrective plan for material failure to meet the prespecified target without good cause.

(d) **Reliable Claims.** A protocol making a causal claim about treatment effects must use randomization or another adequate comparator when feasible and a prespecified analysis. Results without an adequate comparator must be clearly labeled observational and may not be represented as proving causation.

(e) **Public Results.** A qualified evidence system shall publish timely, deidentified aggregate results, including negative and inconclusive results, in a searchable and machine-readable form. Individually identifiable information may not be publicly disclosed.

(f) **Safety Reporting.** Serious and unexpected adverse events must be reported promptly to the supplier, reviewing ethics board, qualified evidence system, and Secretary under timelines established by the Secretary.

(g) **Access and Market Records.** A formal request for treatment under this Act must be

submitted or recorded through a qualified evidence system and assigned a persistent identifier. For every formal request, the system shall record the request date, treatment and condition, patient subgroup characteristics specified in the evaluation protocol, each supplier and provider disposition and stated reason for refusal or withdrawal, price offered, amount agreed and paid, payer category, any reported supply limitation, and whether and when treatment began. A supplier or provider receiving a formal request through the system shall promptly submit its disposition and the required information in its possession, whether or not treatment begins. The system shall link an accepted request to the treatment evidence record, preserve the complete nonpublic records for audit, and provide the Comptroller General access necessary for the evaluation under section 403(c). Public reporting must use deidentified aggregate data and may not disclose individually identifiable information or protected trade secrets.

2.4 SEC. 204. INDEPENDENT ETHICAL AND SAFETY REVIEW

(a) **Review.** Before enrollment, a right-to-trial protocol must be reviewed by an institutional review board under applicable federal law or by an independent ethics review board meeting equivalent minimum standards established by the Secretary.

(b) **Scope.** Review shall address the consent, risk disclosures, monitoring plan, evidence plan, conflicts of interest, and protection of patients unable to consent for themselves.

(c) **Efficient Review.** One reviewing board may approve a protocol used by multiple providers, facilities, or States. Review may be remote. A board may charge a disclosed fee.

(d) **No Treatment Guarantee.** Approval of a protocol does not represent that the treatment is safe, effective, or likely to benefit a particular patient.

(e) **Protocol Suspension.** The Secretary or reviewing ethics board may immediately suspend new enrollment or further treatment under a right-to-trial protocol when credible evidence indicates an unreasonable and significant risk of illness or injury. The Secretary may also suspend distribution under the protocol when necessary to prevent serious harm. The suspension notice must state the grounds and the conditions for resuming the protocol. A provider may continue only the care reasonably necessary to taper, withdraw, or stabilize a patient safely.

2.5 SEC. 205. PAYMENT AND FINANCIAL RESPONSIBILITY

(a) **Negotiated Price.** A willing supplier and patient may freely negotiate payment. The price may include profit and is not limited to direct or indirect cost.

(b) **Price Disclosure.** No patient may be charged more than the amount disclosed at consent unless the patient separately consents to an additional good or service.

(c) **Payment Sources.** A patient, insurer, charity, employer, research sponsor, or other person may pay any portion of the price. Nothing in this Act creates an entitlement to payment or reimbursement.

(d) **Surviving Family.** A patient's heirs or other family members are not liable for an unpaid treatment obligation unless they separately agreed to assume it before treatment.

3 TITLE III: COMPETING EVIDENCE SYSTEMS AND FDA USE

3.1 SEC. 301. QUALIFIED EVIDENCE SYSTEMS

(a) **Qualification.** The Secretary shall publish minimum, technology-neutral standards for the security, privacy, auditability, interoperability, data export, conflict disclosure, safety reporting, and public reporting of qualified evidence systems. The standards must provide protections at least equivalent to the privacy, security, and breach-notification requirements in parts 160 and 164 of title 45, Code of Federal Regulations, and the electronic-record requirements in part 11 of title 21, Code of Federal Regulations, where applicable.

(b) **Open Entry.** A public agency, nonprofit organization, academic institution, or private company may operate a qualified evidence system by publicly certifying compliance with the published standards. The Secretary may audit an operator and revoke its qualification for material noncompliance after notice and an opportunity to cure, except when immediate action is necessary to prevent serious harm.

(c) **Fees.** An operator may charge disclosed fees to suppliers, sponsors, providers, facilities, or patients. Nothing in this Act limits a lawful profit or requires a government-operated platform.

(d) **Portability.** A qualified evidence system must let authorized participants export protocol and evidence records in a documented, commonly usable format.

(e) **No Prescribed Architecture.** The Secretary may not require a particular software vendor, hosting model, source-code license, distributed ledger, artificial-intelligence system, or governance structure.

3.2 SEC. 302. USE OF EVIDENCE

(a) **Fit-for-Purpose Review.** The Secretary may use fit-for-purpose evidence generated under this Act to support approval, clearance, licensure, a new indication, labeling, safety action, or a postmarket requirement.

(b) **Method Neutrality.** The Secretary may not reject evidence solely because a study is pragmatic, decentralized, adaptive, conducted in routine care, or uses real-world data. The weight given to the evidence must reflect its design, data quality, uncertainty, and risk of bias.

(c) **Regulatory Guidance.** Not later than 180 days after enactment, the Secretary shall publish guidance describing how sponsors may use evidence generated under this Act in regulatory submissions.

3.3 SEC. 303. PUBLIC LEARNING

(a) **Interoperable Catalog.** The Secretary shall maintain or designate a public catalog through which patients, providers, and researchers can find registered protocols and aggregate results across qualified evidence systems.

(b) **International Evidence.** A qualified evidence system may accept compatible evidence generated outside the United States when collection and use comply with applicable law.

(c) **No Ownership Mandate.** Nothing in this Act transfers ownership of a patient's identifiable health data or requires public disclosure of trade secrets, manufacturing information, or identifiable health information.

4 TITLE IV: NONINTERFERENCE AND IMPLEMENTATION

4.1 SEC. 401. FEDERAL AND STATE NONINTERFERENCE

(a) **Government Action Prohibited.** No federal, State, or local officer may prohibit or materially burden treatment that complies with this Act solely because the treatment lacks premarket approval, clearance, or licensure for the proposed use.

(b) **Professional Discipline.** A licensing board or government agency may not revoke, suspend, refuse to renew, or otherwise restrict a professional or facility license solely because of good-faith participation in treatment complying with this Act.

(c) **Generally Applicable Protections.** A State may enforce generally applicable professional-licensing, facility, manufacturing, pharmacy, fraud, and safety laws that do not discriminate against eligible investigational treatment or defeat the rights created by this Act.

(d) **Federal Programs.** Participation in this Act alone may not be used to exclude a provider or facility from Medicare, Medicaid, or another federal health program.

4.2 SEC. 402. RESPONSIBILITY AND LIABILITY

(a) **Good-Faith Participation.** A person is not subject to civil, criminal, licensing, or administrative liability solely for good-faith participation in treatment complying with this Act while exercising reasonable care.

(b) **Limits.** Nothing in this Act limits liability for failure to exercise reasonable care, violation of applicable manufacturing standards, fraud, a false or misleading statement, reckless conduct, or willful misconduct.

(c) **Safety Authority.** Nothing in this Act limits the Secretary's authority to investigate a safety signal, require safety reporting, impose or maintain a clinical hold where otherwise authorized, exercise the protocol-suspension authority in section 204(e), or act against conduct outside this Act.

4.3 SEC. 403. IMPLEMENTATION

(a) **Rules.** Not later than 180 days after enactment, the Secretary shall issue rules necessary to implement this Act. The access right in section 201 takes effect 180 days after enactment whether or not final rules have been issued. Until final rules take effect, an evidence system may qualify by certifying compliance with the express requirements of this Act and protections at least equivalent to parts 160 and 164 of title 45, Code of Federal Regulations, and part 11 of title 21, Code of Federal Regulations, whether or not those regulations would otherwise apply to the operator.

(b) **Existing Resources.** The Secretary shall use existing appropriations and may assess transparent, cost-based qualification fees. Nothing in this Act authorizes a new federal appropriation to purchase or subsidize treatment.

(c) **Independent Evaluation.**

1. **Preregistered plan.** Before the access right in section 201 takes effect, the Comptroller General, in consultation with independent health economists, biostatisticians, patient representatives, and drug-development researchers, shall publish a timestamped evaluation protocol and baseline in a permanent public repository. The initial protocol shall remain available. Every later amendment must be logged publicly with its date, reason, and affected analyses. The protocol shall identify outcomes, comparison groups, statistical methods, subgroup analyses, and criteria for interpreting success or failure.
2. **Access and market outcomes.** The Comptroller General shall report annually on requests, enrollments, supplier refusals, posted prices, payments, treatment supply, supplier entry, market concentration, and participation by patient subgroup.
3. **Health and evidence outcomes.** The annual report shall include mortality, condition-specific quality-adjusted life years, patient-reported outcomes, displacement of effective care, serious adverse events, follow-up completeness, missingness, protocol deviations, treatment-condition pairs reaching prespecified evidence thresholds, time to reliable answers, independent replications, and regulatory uses of the evidence.
4. **Methods and uncertainty.** Reports shall distinguish randomized from observational evidence, identify biases and spillovers, publish uncertainty intervals and sensitivity analyses, and release reproducible code and nonidentifiable data to the maximum extent permitted by law.
5. **Economic evaluation.** Public implementation cost, real treatment resources, patient financial burden, treatment prices, medical spending, and net health shall be reported separately. Benefits from earlier access, faster discovery, medical-cost changes, research-cost changes, and option value shall be defined as mutually exclusive channels before aggregation. The evaluation must reconcile interactions and overlap among the channels, and non-additive or overlapping benefits may not be summed.
6. **Counterfactual.** The evaluation shall compare outcomes under this Act with the best available counterfactual, including expanded access, the existing federal Right to Try Act, accelerated approval, off-label treatment, and contemporaneous medical progress unrelated to this Act.

(d) **Severability.** If any provision of this Act is held invalid, the remainder shall not be affected.

4.3.1 End of Act.