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White Paper
Right to Try Legislation
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Purpose

The purpose of this white paper is to concisely summarize the recent "Right to Try" or "RTT" [1] legislation signed into law in May 2018 by President Trump and opine on the potential impact for self-funded payers. The self-insurance industry is presently discussing this new law and its potential impact.

Why the industry is intrigued by this law, given that the FDA's Expanded Access rule(s)[2] have existed since 2009, (which already allow patients to apply for use of unlicensed medication) will be discussed. Benefit plan sponsors and the entities servicing them generally have, in the last 9 years, not asked how said program may affect their plan(s). This paper will seek to delve more deeply into the differences between RTT and existing FDA rules, and identify why RTT has inspired more discussion in a month than the FDA rules have inspired in nearly a decade. Finally, we will examine the new RTT law as an opportunity to be leveraged by benefit plans, and determine what must be done if an employer/group want to cover expenses arising from the RTT law.

In summary, we will address the following:

- How does a plan reliably identify whether complications or side effects are the result of a Phase I drug obtained via the RTT regulations?

[1] Right to Try Act of 2017, S. 204, 115th Cong. (2018)
[2] https://www.fda.gov/NewsEvents/PublicHealthFocus/ExpandedAccessCompassionateUse/default.htm

- Why were the FDA expanded use rules largely unused by patients, and will the RTT be more broadly accessed? Is financial burden a primary reason why the current FDA expanded use program is not utilized, in which case is it fair to say that the RTT law won't have an impact either (unless insurance or plans voluntarily choose to pay for the drug)? Might plan funding for the cost of the drug thereby increase use of the existing FDA program, even without RTT?
- Confirm that there is no legal protection against liability for the benefit plan, an employer, or anyone else that either pays and/or encourages someone to try an experimental drug under RTT. Liability protection is solely for the patient, provider, and drug maker. If a plan does choose to cover such drugs, might the plan need to pursue a hold harmless agreement?
- Additional factors that a plan must contemplate if it wants to extend coverage.

Applicable Legislation

Right To Try (RTT) is a federal law, which presents an alternative for eligible individuals to seek drug coverage for treatment options which have only passed Phase I clinical trials. To gain access to these drugs, a terminally ill patient's physician must approve the experimental drug and ask the drug manufacturer for patient approval. The drug manufacturer does not have an obligation to grant access. If the manufacturer approves, upon approval the drug manufacturer must grant access to the drug to the patient at the actual cost. Insurance companies and/or benefit plans are not required to cover the experimental drugs under the RTT law or the side effects. Under RTT a patient remains responsible for the payment of the treatment, unless the patient's plan otherwise decides to cover the cost.

In summary, the law does include the following:

- Allows terminally ill patients with physician approval to request access to experimental drugs which have completed Phase I clinical trials;
- Protects manufacturers/physicians from liability stemming from such use; and,
- Allows the manufacturer to bill patients for the cost of the drugs.

What the law does *not* do is the following:

- Require a manufacturer to provide any drug (i.e. it may, as was the case before, reject any such request); or,
- Require any payer/insurer to cover any drug or side effect.

Brief Analysis

The actual change from existing "expanded access" or "compassionate use" guidelines will likely be minimal for patients and manufacturers. From a payer standpoint, this legislation has no direct impact at all, however the potential for increased access to experimental and/or investigational drugs highlights the importance of strong plan language, as well as identifying and closing gaps in relation to stop-loss coverage.

Specifically, a self-funded plan wishing to voluntarily include coverage for these experimental drugs under the RTT law will need to modify certain provisions in their plan materials (i.e. Experimental and Investigational language) and discuss such changes with their stop loss carrier to ensure reimbursement is available (and if parameters apply, what might those prerequisites be), and eliminate coverage gaps. Reasons a plan sponsor may seek to provide coverage include, but are not limited to, altruism and a desire to facilitate any and all opportunities to cure a terminally ill participant, as well as potential cost saving. Indeed, a terminal patient may be taking a regimen of approved drugs, that the plan is required

to cover at or close to 100% of the cost, and if an experimental drug secured via RTT results in expedited healing, the amount of covered (licensed) treatment will thereby be limited, resulting in net savings to the plan. Note that the drug manufacturer, under RTT, may only charge the actual cost. Further it has been suggested that a plan may make payment for an RTT drug contingent upon the patient ceasing use of an otherwise licensed, covered drug; (imagine if a plan agrees to pay for 50% of an experimental drug in lieu of a licensed and approved drug for which they are required to pay 100%). This last scenario, however, is not advisable, as – should the patient suffer permanent injury or death (and if one could argue that the treatment that was discontinued to secure payment for the RTT treatment may have prevented the damage or death) – the entity that "forced" the patient to cease treatment may be deemed liable.

Discussion

In late May, Congress passed the RTT law which grants access to non-Food and Drug Administration (FDA) approved experimental drugs to certain individuals. Specifically, RTT gives patients with life-threatening diseases (who have otherwise exhausted all other approved treatment methodologies and are not eligible for, or cannot enroll, in a clinical trial) the right to seek experimental drug treatments (i.e. those that passed Phase I of the FDA approval process, but are not fully FDA approved).

Existing Expanded Access and Similar Programs

Currently, 38 states (Alabama, Arizona, Arkansas, California, Colorado, Connecticut, Florida, Georgia, Idaho, Illinois, Indiana, Iowa, Kentucky, Louisiana, Maine, Maryland, Michigan, Minnesota, Mississippi, Missouri, Montana, Nevada, New Hampshire, North Carolina, North Dakota, Ohio, Oklahoma, Oregon, Pennsylvania, South Carolina, South Dakota, Tennessee, Texas, Utah, Virginia,

West Virginia, Washington and Wyoming) have passed similar legislation, but RTT will introduce legislation across state lines.

In addition to the legislation in those 38 states, the FDA does already have an expanded access (or 'compassionate use') policy which grants terminally ill patients access to experimental drugs. Pursuant to the existing FDA expanded access program, the requirements for all "Expanded Access Uses" include:

1. The patient and a licensed physician are both willing to participate;
2. The patient's physician determines that there is no comparable or satisfactory therapy available to diagnose, monitor, or treat the patient's disease or condition;
3. The patient is unable to obtain the investigational drug via another program or to participate in a clinical trial;
4. The probable risk to the person from the investigational product is not greater than the probable risk from the disease or condition;
5. The FDA determines that there is sufficient evidence of the safety and effectiveness of the investigational product to support its use in the particular circumstance;
6. The FDA determines that providing the investigational product will not interfere with the initiation, conduct, or completion of clinical investigations to support marketing approval;
7. The sponsor (generally the company developing the investigational product for commercial use) or the clinical investigator (or the patient's physician in the case of a single patient expanded access request) submits a clinical protocol (a document that describes the treatment plan for the patient) that is consistent with FDA's statute and applicable regulations for INDs

or investigational device exemption applications (IDEs), describing the use of the investigational product; and

8. The FDA must determine that the potential patient benefit justifies the potential risk of the expanded access use of the investigational drug, and that the potential risk is not unreasonable.

The RTT legislation in essence bypasses items 5 through 8.

The FDA's official guidance also states, as it relates to the existing FDA expanded access program and "Investigational Product Availability and Costs" in particular:

- The medical product company must agree to provide the investigational drug for expanded access use. FDA cannot require a company to provide an investigational drug for expanded access use to proceed.
- A company may decide to turn down a request if, for example it is not able or willing to provide access to an investigational drug outside of clinical trials intended to support marketing approval.
- In some cases, patients may have to pay for using the investigational drug and/or for medical care associated with the use of the investigational drug. In others, pharmaceutical companies may elect not to charge.

Under the current legislation governing these programs, neither health insurance nor benefit plans are required to pay for all or some of the cost. Nothing, however, prohibits them from doing so either.

Current Federal Guidance on Existing Expanded Access Programs

The official FAQ from a combined HHS/DOL/FDA on the matter[3] (existing FDA expanded use programs) provides the following in relevant part; [note the word "sponsor" refers to the drug maker]:

> Q17: What costs can a sponsor recover when charging for an investigational drug for expanded access use under 21 CFR part 312, subpart I?
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> A17: When charging for individual patient expanded access (under § 312.310) to an investigational drug, a sponsor may recover only its direct costs associated with making the drug available to the patient (see Q1 6 and § 312.8(d)). For individual patient expanded access, the sponsor may not charge for indirect costs, including administrative costs associated with providing an investigational drug. Examples of indirect costs include -
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> • Costs associated with developing the treatment protocol and informed consent document
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> • Costs associated with corresponding with the IRB, FDA, and/or the drug manufacturer
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> • Costs associated with reporting to the IRB and/or FDA
>
> • IRB fees and expenses
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> Q3: Once FDA authorizes a request to charge, whom may the sponsor charge?
>
> A3: Although FDA determines whether a sponsor may charge for an investigational drug used in a clinical trial or for expanded access, FDA does not decide how that charging is to be carried out. FDA anticipates that the sponsor would ordinarily charge a patient directly or would charge a third-party payer if reimbursement were available. FDA notes that it has no authority to require that the Centers for Medicare and Medicaid Services reimburse for investigational drugs

[3] https://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm351264.pdf

for which FDA has authorized charging. Similarly, FDA has no authority to dictate reimbursement policy to any other entity, including private health insurance providers. For questions pertaining to third-party payer reimbursement, the third-party payer should be consulted.

Q20: What information is a sponsor required to submit to support its cost calculation?

A20: Under 21 CFR 312.8(d)(3), to support its calculation of recoverable costs, a sponsor must provide documentation to show that its calculation is consistent with the requirements of § 312.8(d)(1), describing recovery of direct costs and, if applicable, the requirements of § 312.8(d)(2), describing certain additional costs that may be recovered for intermediate-size patient population expanded access uses or treatment INDs or protocols. This documentation must be accompanied by a statement that an independent, certified public accountant has reviewed and approved the calculations (§ 312.8(d)(3)).

The law itself says - "Right to Try Act of 2017 - This bill requires the federal government to allow unrestricted manufacturing, distribution, prescribing, and dispensing of experimental drugs, biological products, and medical devices that are: (1) intended to treat a patient who has been diagnosed with a terminal illness, and (2) authorized by state law. The federal government must allow unrestricted possession and use of such treatments by patients certified by a physician as having exhausted all other treatment options. A manufacturer, distributor, prescriber, dispenser, possessor, or user of such a treatment has no liability regarding the treatment."

Concerns with Existing Programs

As mentioned, despite the similar RTT legislation that is available in 38 states, and the FDA expanded access policies, very few individuals have taken advantage of these programs. Many believe the new RTT program is not in the best interest of patients due to the weakened FDA oversight, while others question the need for RTT, in light of the very limited use of the aforementioned existing FDA Expanded Access program.

Potential considerations as to why the current programs are not being utilized may relate to: (1) the elements of the existing program that provide the FDA with oversight (an added level of "red tape"), (2) fear on the part of providers and manufacturers regarding legal liability if things should go poorly, and (3) the burden for payment resides solely with the patient.

Regarding FDA "red tape," a key difference between the RTT rules and existing FDA expanded access rules are that previously, such a request would have to go through the FDA for approval, but the FDA has reported that it approved 99% of such applications, so the actual practical difference in regard to patient access with and without FDA approval will likely be very minimal inasmuch as said "red tape" is concerned.

Are The Differences with the New Right To Try Legislation Enough to Increase Usage?

The new RTT legislation may potentially lessen two of these three considerations (bureaucracy and liability) and increase access by patients as it relates to cost (due to a provision limiting cost of the drugs to the actual cost).

Bureaucracy: Unlike existing "expanded access" or "compassionate use" guidelines which involved FDA oversight, the new RTT legislation will allow terminally ill patients to bypass the FDA and, with

physician approval, gain access to drugs which have completed a Phase I clinical trial but are not otherwise FDA approved for use, either in general or for the condition in question.

Liability: The law protects both the manufacturer and the referring physician from liability stemming from use of such experimental/investigational drugs. Note that the protections available under the RTT regulation do not extend to the insurers or plans, so entities wishing to provide coverage for RTT drugs should consider the potential liabilities that could exist in doing so and/or consider a waiver or hold harmless agreement.

Implications for Self-Funded Plans

As noted above, the law does not impose any requirements on payers to cover drugs accessed through this law, however, as always, it is crucial for plan sponsors to ensure that they have strong plan language and verbiage explicitly matching plan intent. Limitations on claims resulting from side effects and/or complications from use of these drugs, if said use is a non-covered treatment, will be important – such charges could easily exceed the cost of the drug itself.

Finally, if a plan does determine to extend coverage for drugs under the RTT regulations, it is important for plan sponsors to examine the applicable stop-loss and/or reinsurance policies for any gaps in coverage in regard to experimental/investigational treatment, and in regard to complications stemming from non-covered charges.

Recommendations

Self-funded plans wishing to ensure drugs under the RTT law are not covered

1. Defined Terms. Review the definitions section to ensure all references to drugs are those which are FDA-approved. For example, tie the definition of drug to an FDA approved drug and/or

consider a caveat that drug shall not mean a drug which has only passed a FDA Phase I clinical trial. In addition, within the definition of experimental and/or investigational ensure that it complies with the ACA clinical trial regulation (if non-grandfathered) but that Phase I drugs are considered experimental. Remember that drugs received via the RTT law are not part of an approved clinical trial.

2. Excluded Benefits. Review the exclusions and ensure there is one for experimental and/or investigational treatments. In addition, consider whether the plan wants to cover the side effects from an RTT drug and if not be sure to add a caveat (and/or add) to the exclusions, complications from non-covered services. While not necessary, a concerned plan could also add a specific RTT exclusion.

Self-funded plans wishing to ensure drugs under the RTT law are covered

1. Defined Terms. Review the definitions section and add a caveat regarding Phase I drugs obtained via the RTT legislation by eligible individuals (i.e. definition of covered drugs and carve them out from the definition of experimental and/or investigational). Be careful with how this is modified in relation to the ACA clinical trial language (drugs obtained via RTT are not part of an approved clinical trial).
2. Excluded Benefits. Review the exclusions and add a caveat to the experimental and/or investigational treatments provision stating that drugs obtained by eligible individuals via the RTT law are not excluded. In addition, consider whether the plan wants to cover the side effects from an RTT drug's usage and if not, be sure to add a caveat (and/or add) to the exclusions provision, complications from RTT drug usage.

3. Covered Benefits. Review the covered benefits section and consider adding a specific benefit for drugs obtained via the RTT law by an eligible individual. This provision should clarify whether the plan will cover side effects or complications from the RTT drug. Pursuant to the regulations, coverage for the drugs and the side effects are not required, so plans will have the opportunity to be flexible with coverage (i.e. cover the drug only and/or cover the side effects only or both); adjust the percentage covered, etc.
4. Utilization Management. The implications for how and the extent to which coverage may be provided under the plan should be discussed with the current utilization management company or other pre-certification vendor as certain clinical aspects should be investigated (i.e. identification of an illness or condition as a side effect of the particular drug).
5. Schedule of Benefits. Review the schedule of benefits and consider adding a new line item for this covered benefit or benefits. Since coverage is not required and is not an essential health benefit, employers may place dollar limits, pre-certification and/or other cost-sharing requirements. For example, the plan may wish to partially pay or pay a percentage of a capped amount; regardless of the 'actual cost' charged by the manufacturer. We stop short, however, of making coverage contingent – in whole or in part – upon ceasing another FDA approved and otherwise covered treatment(s). The reason for this limit, is that should the patient suffer permanent injury or death, and one could argue the treatment that was discontinued to secure payment for the RTT treatment may have prevented the damage or death, the entity that "forced" the patient to cease treatment may be deemed liable.

6. Stop Loss Considerations. Prior to implementing the benefit modification, ensure the ERISA regulations for timely notice are followed and that stop loss is aware and willing to reimburse claims (i.e. close the potential gap in coverage).

Action Items

For additional assistance or consultation regarding plan design modifications or review to ensure current plan language is consistent with the plan sponsor's objectives relative to the Right To Try legislation, please contact Phia Group Consulting, LLC at pgcreferral@phiagroup.com

Disclaimer: This opinion is based on the facts as presented and re-stated above, and our research, which includes the sources utilized as of the date this opinion has been drafted. This is a consulting opinion only, and does not purport to offer legal advice or fiduciary guidance as to the denial or acceptance of claims or appeals. This opinion is based upon our interpretation of the relevant materials and may not conform to official interpretations of statutes, regulations, contracts, or other materials. Subsequent changes in applicable law may change the result of this opinion. Ultimately the Employer/Plan Sponsor is responsible for making all business decisions related to their benefit offerings (including drafting and ratifying its plan documents) and, as the Plan Administrator, has the discretionary authority to interpret the terms of the Plan Document and make benefit determinations.