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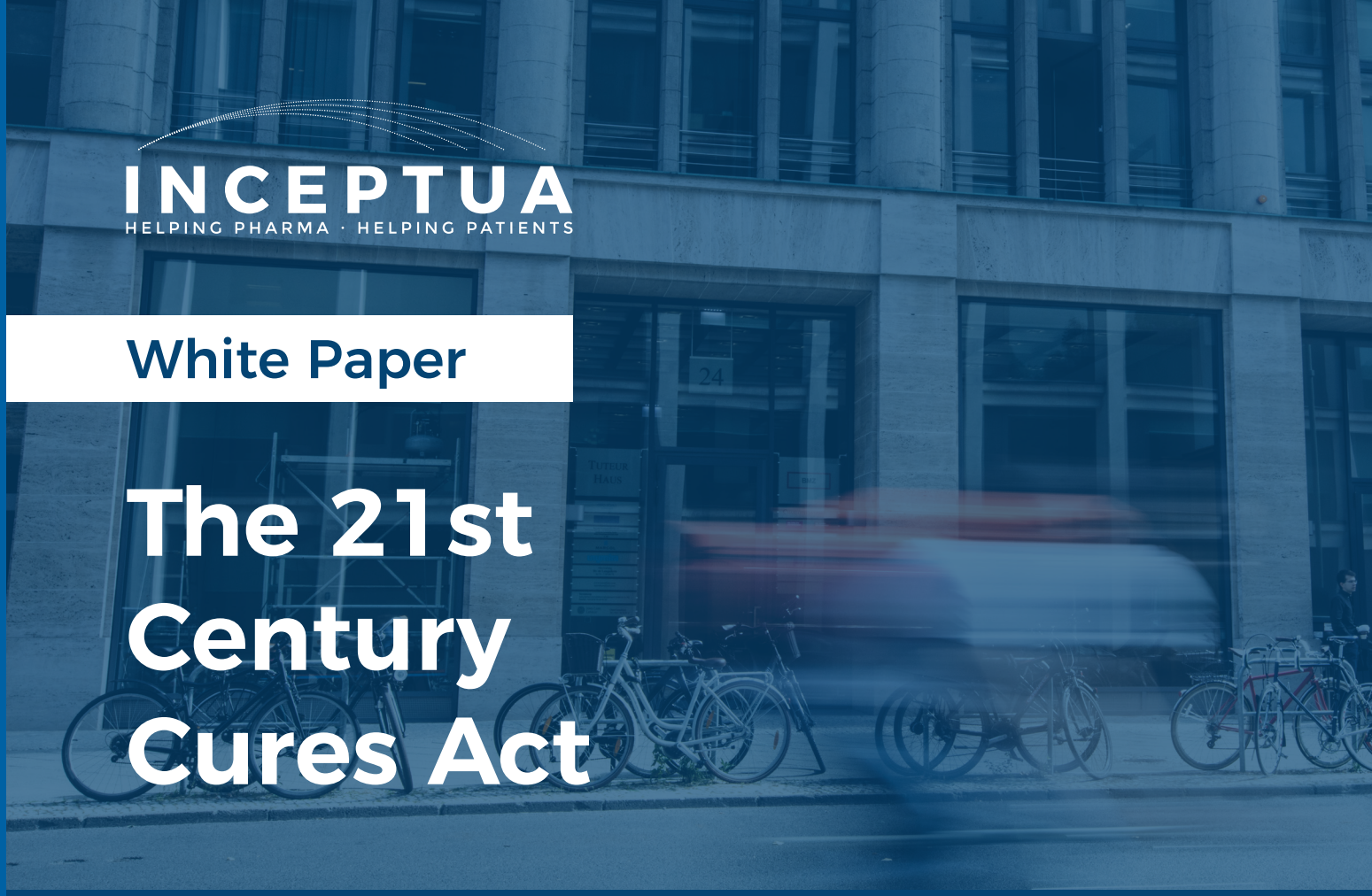
White Paper

The 21st Century Cures Act



Turn your obligations into an advantage

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The 21st Century Cures Act: Turn your obligations into an advantage

The 21st Century Cures Act and Expanded Access

The 21st Century Cures Act (21CCA), which came into effect in February 2017, is intended to help accelerate the development of medicines and bring those medicines to patients more quickly. One aspect of the 21CCA requires that manufacturers of one or more investigational drugs for the diagnosis, monitoring, or treatment of one or more serious diseases or conditions must have a readily-available policy on expanded access (also known as pre-approval access, early access, named patient supply) to their medicines on a publicly available website.

Although the 21CCA is directly relevant only to companies with a USA presence, its impact has been felt across the world, due to the attention it has brought to the wider issue of earlier access to medicines, especially the staggered approvals process in Europe which can see physicians in one country prescribing a licensed medicine, whilst their colleagues only a few miles away in another country may have to wait many more months, or even years, to access the same drug through the commercial route.

In October 2016 Avalere Policy 360° conducted research which showed that of 100 pharma and biotech companies reviewed, only 19% had a public policy on pre-approval access at that time [1]. A follow-up analysis conducted by Avalere in March 2017 (one month after the enactment of the 21CCA) showed that this number had increased to 48%. When this was broken down into large, medium and small companies the percentage with public policies was 84%, 52% and 24%, respectively (Figure 1). Since then, data is scarce as to what percentage of pharmaceutical and biotech companies have such a policy. The data show that large pharma are leading the way with regard to access policies, but it is clear that many medium- and small-size pharma and biotech have yet to consider pre-approval access at least in any way they are willing to make public.

Figure 1: Percentage of Companies That Post Compassionate Use Policies on Their Websites

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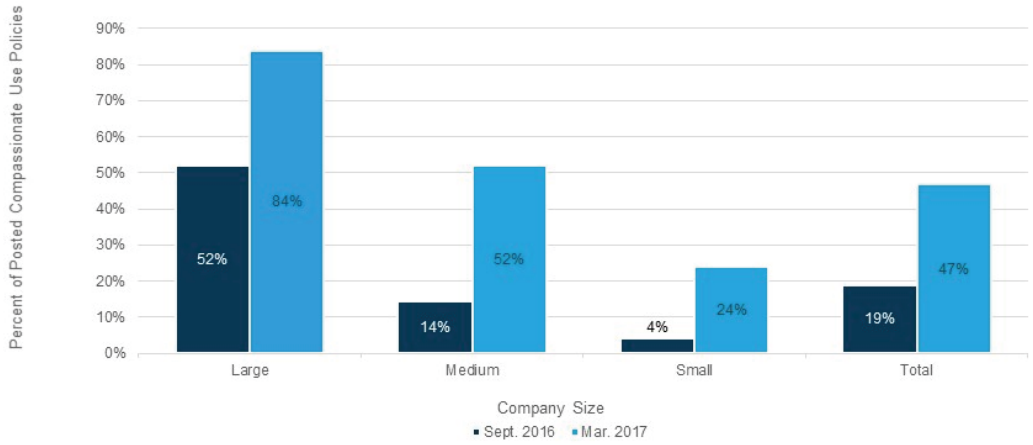

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The Reagan-Udall Foundation launched its 'Expanded Access Navigator' in August 2017, with the intention of increasing the understanding of the request process for access to investigational medicines, and to provide a repository for companies to list their pre-approval access policies. Pharmaceutical manufacturers are encouraged to post summary information about their pre-approval access activities, and a link to their policy on pre-approval access in a searchable database within the Navigator. As of August 2019, the Expanded Access Navigator listed 65 pharma and biotech companies pre-approval access policies. It is likely that not all companies with policies have listed them in the Navigator, but even so, such a relatively small number listed in such a high-profile database suggests that many companies do not have such a policy at all.

Although there are no publicly-available examples of manufacturers being reprimanded for not complying with the pre-approval access policy requirement of the 21CCA, it is clearly in a company's best interest to comply with this legislation, from a legal compliance perspective, but also to ensure that they have considered how they will approach pre-approval access requests, in advance of actually receiving any such requests. This policy is a key piece of patient engagement, and should be accorded some attention, particularly if you are operating in the rare disease space, or in the situation where you are a small biotech with only 1 asset.

The reasons being, in the rare disease space, patients will be following your clinical development/publication of results closely, and will be keen to access your product as soon as it shows efficacy, and a carefully-constructed policy is crucial to ensuring that a) patients can easily find information and b) that the messaging and tone do not risk their disenfranchisement if your decision is not to provide pre-approval access. For small biotechs, a single-asset company can be more at risk of negative reactions if the complexities of pre-approval access are not explored in advance of requests, and a well-thought out stance adopted – Chimerix is a good example of the potential pitfalls around pre-approval access, in situations where emotions are running high and patients are desperate to access a particular medicine [2].

What are the possible solutions?

With no apparent sanctions for not having a pre-approval access policy, the path of least resistance is to not develop one. Reasons for this could include, a lack of awareness of the need to implement, not considering it a priority, not understanding what might be required, not having an established position on pre-approval access, not wanting to commit resource to responding to queries – a requirement of the Act is to provide a point-of-contact and a commitment to a response time.

The majority of company policies on access which are in place, are fairly straightforward and simplistic. It is perfectly feasible to develop a standalone policy, written in isolation, or from reusing parts of existing policies. However, while it is possible to write a brief policy satisfying the key requirements, with some specifics related to your organization as a standalone piece, this approach runs the risk of being either too vague or too restrictive, if a position on pre-approval access is adopted without wider discussions of the pros and cons and potential impact on corporate reputation, involving all internal pre-approval access stakeholders e.g. medical, supply chain, regulatory, finance and legal.

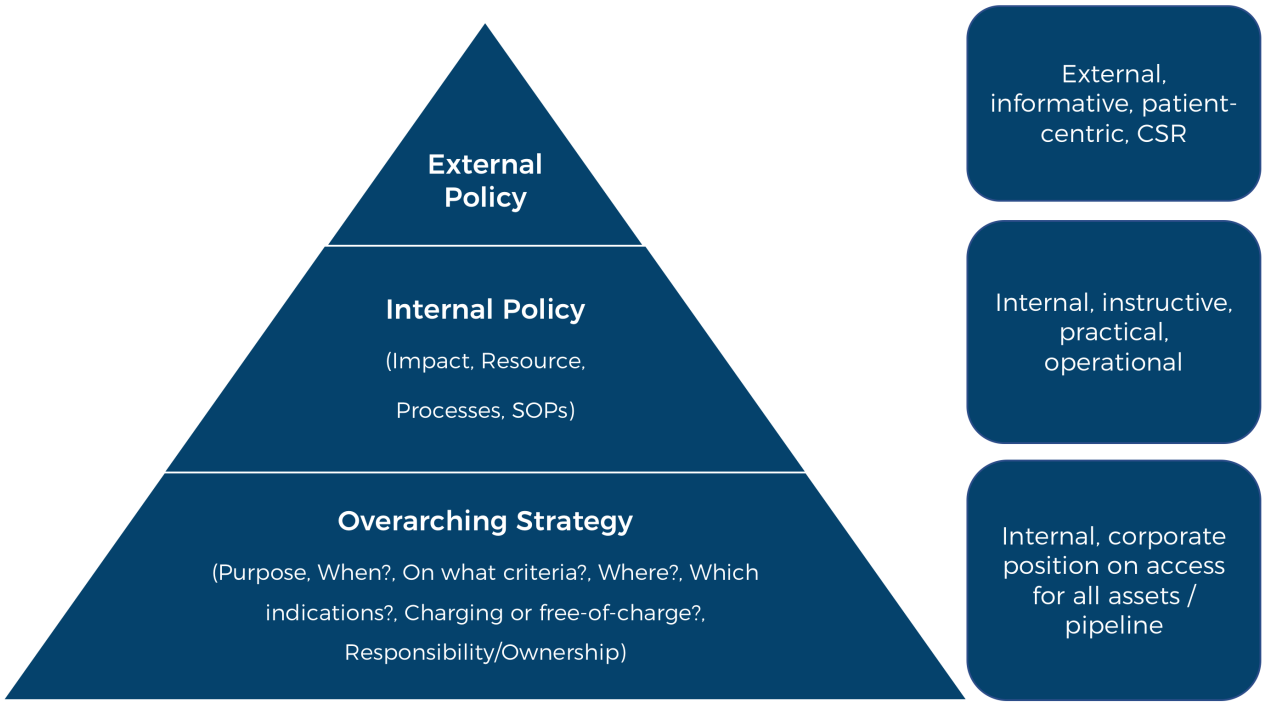
It is important that any policy is clear, no longer than strictly necessary, is readily findable on your website and is constructed with, if not patient advocacy input, then certainly based on some interactions with your patient community. There can be a temptation to include too much detail not directly relevant to pre-approval access in a policy. Patients seeking information about whether/how they can access a medicine want to find that information readily, so it is best to avoid detailed descriptions of your clinical trial process, or any other information not directly pertinent to your stance on pre-approval access.

There is a risk that such policies are written from too much of a corporate/commercial perspective. Care should be taken to avoid this.

An alternative approach is to view the policy as the final, patient-centric output of a much broader piece of work, designed with the intention of defining your corporate ethos on pre-approval access.

How can you use the 21CCA to enhance your reputation and support patients?
Superficially it might seem like overkill, but the most robust approach to developing a public policy on pre-approval access is to start first with a detailed corporate strategy on pre-approval access (Figure 2.). This may sound like stating the obvious, but every company has a unique corporate ethos and that ethos dictates its approach to pre-approval access. Some companies have the intention of reaching every patient with the condition anywhere in the world. Others will only provide access in countries where they intend to commercialize. Some will provide access as early as PII, others only after FDA approval. Some will provide product free-of-charge, whilst others will only charge for access. The approach taken depends on a number of important corporate decisions.

Figure 2. Recommended Structure for Development of a Public Policy on Access



The unifying, common principle across all companies, with regard to pre-approval access, is a commitment to patient access and an ethical imperative to 'do the right thing'. Beyond those core intentions, there are many other factors at play. Larger pharma companies have been known to initiate access programs with the intention of getting to market ahead of a competitor's launch, whilst smaller companies and biotechs often use this approach to 'test the waters' in Europe to guide commercial capacity-building. Every company's objectives are different, and so the corporate strategy must be customized to each company and potentially each asset. Once this corporate strategy is developed, then it is relatively straight forward to take that and develop it into a detailed internal policy on access from which, ultimately, the patient-centric public policy should be derived.

A standalone policy is at risk of not taking into account the wider context and implications of pre-approval access, if it is not based on a much more detailed understanding of a company's ultimate objectives in providing pre-approval access to its medicines.

Committing to pre-approval provision of a medicine raises many issues such as how to transition to commercial supply, how to transition to charged-for supply if you commenced with an initial free-of-charge pre-approval approach? Longer range considerations include, what will your approach be if your PIII does not read-out as anticipated?

If you have not considered under what circumstances you will provide pre-approval access you may prefer to kick the can down the road until you are actually receiving demand from patients and physicians. But in the long-term, taking steps now to define your corporate ethos on pre-approval access will make the development of a public policy on access much easier and will ensure you are in a position to reply to requests in a coherent manner, with the knowledge that all key stakeholders have a full understanding of your approach, and the rationale/justification for that approach.

References

1. Avalere Policy Preapproval Access - Exploring the Landscape for Compassionate Use, Expanded Access, Right-to-Try, and How Companies can Prepare and Take Action. February 2017.

2. Ethical crossroads: expanded access, patient advocacy, and the #SaveJosh social media campaign. Moch, I.K. Med Access @ Point Care 2017; 1(1): e119-e130.

About the author: Stuart Bell, Ph.D., Vice President, Consulting, Inceptua Medicines Access.

With over 20 years of industry experience, Stuart Bell leads the consulting arm of Inceptua's Medicines Access business.

Stuart Bell is an experienced pre-approval (compassionate use / expanded access) specialist with a strong track record in commercial consulting with respect to global access strategies, feasibility studies, launch planning, real-world data capture, and market access, with a core focus on rare diseases, orphan products, and oncology.

Stuart Bell obtained his Ph.D. degree in 2001 from the University of Stirling.

About Inceptua

Inceptua is a pharmaceutical company and service partner spanning throughout the product lifecycle – from comparator sourcing for clinical trials, through early access programs to licensing and commercialization of products. We partner with life science companies of all sizes, drawing on over 20 years of industry experience.

Our offering includes registration and commercialization of products through in-licensing and flexible partnerships, and the we have leading expertise in strategy and operational implementation of pre-approval access programs making pharmaceutical products under clinical development available for patients. Our clinical trial services business offers high quality clinical comparator sourcing and manufacturing services with an agile global supply chain to ensure that products are delivered exactly when needed.

Inceptua has global operations with local offices across Europe, USA, and Asia.