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# **How can health research regulation better support access to experimental or innovative medical treatment?**

## **Policy brief**

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**[www.law.ed.ac.uk/research/research-projects/liminal-spaces](http://www.law.ed.ac.uk/research/research-projects/liminal-spaces)**

## Overview

Patients who have exhausted all conventional or existing medical treatments may seek to access an experimental or innovative treatment as a last best hope. This is not always easy. This brief looks at how healthcare regulation could better support access to experimental or innovative treatments.

This document draws on research by the [Liminal Spaces Project](#) at Edinburgh Law School, a six-year Wellcome-funded project that examined health research regulatory systems and how their operation can be improved.

## What is experimental treatment?

A medical treatment is considered **experimental** if:

- It is a new, unknown, or rarely used intervention, and there is uncertainty about safety and efficacy because of a lack of evidence
- It does not conform to usual clinical practice supported by medical practitioners
- It is currently undergoing or has yet to undergo clinical trials
- It has not received approval from a relevant regulatory body, e.g., the UK's [MHRA](#).

A medical treatment is generally only approved for specific purposes. A treatment can also be considered experimental where it is used for different purposes to those originally approved. This is called '**off-label**' use. Experimental treatment aims to benefit an individual patient or small group of patients. This makes it different from a clinical trial, which aims to generate knowledge about a new drug or vaccine at the population level, i.e. is it safe to bring to market for general consumption?

## The regulatory environment for experimental treatment

'Experimental treatment' sits somewhere between established medical care and novel health research. Experimental treatment often proceeds on a case-by-case basis, trying to find a course of action that benefits the individual patient. In regulatory terms, this causes many problems. First, if experimental treatment is neither standard care nor protocol-driven research, then it is unclear which regulatory environment governs such practices. Second, because much experimental treatment is explored patient-by-patient, there are few regulatory mechanisms to capture what counts as good evidence.

On this last point, the [Access to Medical Treatments \(Innovation\) Act](#) (2016) seeks to promote access to **experimental or innovative** treatments and supports the creation of a database of innovative treatments.

Other mechanisms have also been introduced, such as:

### **‘Specials’**

Patients with a real clinical need may gain access to unlicensed medicinal products called ‘[specials](#)’ through the Human Medicine Regulations 2012 regulation 167. A ‘special’ is an unlicensed medicinal product that has been specially manufactured or imported to the order of a prescriber. These products are only supplied to individual patients with special clinical need.

An example of a ‘special’ is [unlicensed cannabis based products for medicinal use](#). In order to access these products in the UK, a special licence must be sought from the Home Office.

[The Early Access to Medicines Scheme](#) (EAMS) grants patient with life-threatening or serious conditions access to medicines that have not yet received a licence or marketing authorisation if there is a clear unmet medical need. One example is [atezolizumab](#) (Tecentriq), a treatment for liver cancer given to over 600 UK patients with limited options before licence approval.

## **The proposal for a database for innovative treatments**

The [Access to Medical Treatments \(Innovation\) Act](#) (2016) is a very short piece of legislation that gives a general overview of innovative treatments as those that depart from the existing range of existing medical treatments for the condition in question. However, it does not fully expand on this definition. It also seeks to establish a database of innovative treatments with the intention of allowing seriously ill patients to volunteer for innovative treatments. Such a database would allow doctors to see which treatments had been offered and with which effects. Section 2 of the Act states that this database is to be maintained by the Health and Social Care Information Centre (HSCIC). However, to date, this database has not been established, nor does the legislation state which agency has the ultimate authority for it. Furthermore, the Act gives no indication of how access to this database should be granted, nor who should be responsible for the operation and updating of such a database once it is created, e.g. what would be the responsibilities, if any, at the healthcare practitioner level to provide evidence of experimental practices?

**Recommendation:** The establishment of such a database requires clear lawful authority and social licence for its operation. Seeking social licence is more than merely passing legislation; it requires demonstrations of transparency, accountability and engagement with all stakeholders –

including the public – in all actions, especially where this might involve the use of patient-level data.

The database of innovative treatments, once established, must be easily accessible and user-friendly for patients and healthcare professionals who wish to access its data, or contribute new data. Such easy access could be supported by guidance notes or [regulations to follow](#) the Act.

Without proper oversight and accountability, there is a serious risk that such a database could be counter-productive in encouraging needlessly risky interventions.

## **Navigating the complex regulatory environment(s)**

The regulation of access to experimental treatments is difficult to navigate, not least because the Access to Medical Treatments (Innovation) Act does not specify how experimental or innovative treatment is to be regulated or accessed. But this legislation sits alongside a morass of other laws. For example, although the [Human Medicines Regulations 2012](#) regulate the use of medicinal products for human and veterinary use, it also does not define 'experimental treatment'. Also, do the laws that apply to standard care simply stop applying to experimental interventions that go beyond the bounds of what is generally accepted or must healthcare practitioners navigate both the rules of care and research?

All of this means that any new attempts to deal with the regulation of experimental treatments can result in overlap, duplication of information, or increased confusion in trying to interpret all the many regulatory instruments.

Taking a whole system approach to these issues would allow researchers, practitioners, and regulators to see that experimental treatment is a complex area that straddles a number of regulatory categories. The Liminal Spaces project [has explored elsewhere](#) what it means to take a whole system approach, particularly where multiple regulatory systems operate.

The Liminal Spaces Policy Brief on the challenges of [uncertainty](#) in health research recommends the use of [regulatory stewardship](#) to help navigate and access difficult areas of health regulation. Regulatory stewards are people familiar with regulatory requirements and give support across regulatory complexities where such uncertainties arise. This helps to reduce regulatory burdens and create more opportunities for both researchers and those wishing to gain access to experimental treatments. Regulatory stewardship is particularly helpful in areas such as experimental or innovative medical treatment as it involves both the activities of treatment and research.

**Recommendation:** A model of regulatory stewardship under the auspices of a relevant regulatory agency – such as the Department of Health and Social Care in England - could form the basis of guiding action for healthcare professionals and patients to navigate the complexities

of providing experimental treatments safely and in a timely fashion, while also helping to gather evidence of what works and what does not work.

## **Blurred boundaries between research and treatment**

As is clear from the above, experimental treatment exists in a space that [blurs the division](#) between treatment and research. Although it provides therapeutic benefit to individuals or small groups of patients, its innovative approach means that it also has the potential to contribute evidence to assess which interventions do or do not 'work'. This means that it can be difficult to see where treatment ends and research begins. In addition to the complexities mentioned above about how experimental treatments are regulated, this can lead to confusion around who is liable if an experimental treatment goes wrong. Is a healthcare professional to be negligence for trying something new and/or should a drug manufacturer escape all liability if their product is used in unanticipated circumstances?

This is not to say that experimental treatment should be subject to categorisation as either an instance of 'treatment' or 'research'. This could lead to practices being under- or over-regulated. The effects of either extreme would be significant. If practices are over-regulated, this could lead to lack of a flexibility on the part of healthcare practitioners, researchers and patients to access these treatments or processes. If they are under-regulated, there could be insufficient protection for patients, thus risking unwarranted harm to them.

It is for this reason that the Liminal Spaces project has highlighted the importance of a ['processual'](#) approach to regulation. This allows for more flexibility and appropriately tailored regulation whereby there is sharing of information across the whole systems based in health regulation. Adopting a processual approach to experimental treatment would recognise that this practice straddles two areas that are regulated in different ways. Processual regulation involves the identification and full use of ['feedback loops'](#) on what works and what does and does not work to the actors within a regulatory system to make changes as are necessary. Focussing on the creation of effective feedback loops – rather than concerns about potential liability – can help to create a positive and supportive culture of mutual learning and social value.

### **Recommendations:**

To establish an optimal regulatory system for experimental treatments, policymakers and regulatory agencies should adopt a whole [systems](#) approach to experimental treatment that allows practitioners, researchers and regulators to recognise that experimental or innovative medical treatment cannot be compressed into one single category of regulation. By adopting a whole systems approach that is flexible and inclusive – especially in gathering evidence of what works well and less well – the regulatory ecosystem could promote the working together of patients, practitioners, researchers, and regulators. Such a coordinated approach would both

promote valuable experimental treatments and help to protect against those that are useless or potentially harmful.

A regulatory culture that is driven by fear of potential liability promotes caution and concern. Options for reform in this area should therefore not begin by addressing liability concerns – they should promote sharing of best practices and provide support mechanism to capture emerging evidence around all kinds of innovative treatment.

## Sharing knowledge, results and outcomes

Because experimental treatment straddles the boundaries of treatment and research, it is important that knowledge gained in the course of providing an individual benefit is shared. Any knowledge gained through administration of experimental treatment is of value to health research. But, there is also a lot [uncertainty](#) generated by the use of novel, unproven, or untested treatments and therapies. This gives both practitioners and researchers the opportunity to participate in **feedback loops** – a continuous learning cycle from uncertainties that affect both research and practice.

**Recommendation:** Those involved in research and the provision of experimental treatment should identify, publish and share all lessons learned, both positive and negative. Lessons are learned from mistakes as well as successes. Administration of experimental treatment should be seen as an opportunity to further both individual and societal benefits as it generates generalisable knowledge for health research. Promoting and sharing knowledge gained also increases transparency, research integrity and can support public [trust](#) in health research.

## Conclusion

The regulation of experimental or innovative medical treatment is a complex area that does not exist in one single regulatory silo. There are many areas and pieces of regulation that deal with this field. It is a practice that straddles the activities of both treatment and research. Regulatory stewardship is encouraged to help navigate this complexity, which should be seen as a valuable opportunity for feedback loops to generate knowledge both for the individual wishing to access this treatment, and for society. Approaching the two practices of treatment and experimentation separately could lead to disconnection and missed opportunities to learn from each other. Active collaboration across these fields will increase shared knowledge in health research and this can best be achieved by adopting a whole system approach.

## **Acknowledgments**

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## **Read more about Liminal Space's work:**

More information can be found on the [Liminal Spaces project legacy website](#).

Sethi N. (2019). Regulating for uncertainty: bridging blurred boundaries in medical innovation, research and treatment. *Law, Innovation and technology*, 11(1), pp. 112-133.

[Regulatory Stewardship in Health Research](#)

[Processual regulation in health research](#)

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