

Nordic Committee on Bioethics  
*Bioethics of clinical innovation and unproven methods*  
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# Novel medical treatments: innovation, hope and headlines

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# BIOETHICS BRIEFING NOTE

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## Patient access to experimental treatments



### OVERVIEW

- Patients might access experimental treatments if other treatments have not worked or are not available.
- There are several ways in which experimental treatments can be offered legally to UK patients, or patients may travel abroad to access treatments not offered in the UK.
- The use of experimental treatments can raise ethical issues such as: difficulties in assessing efficacy and safety; ensuring fairness of access; challenges around decision making and consent; potential impacts on knowledge generation; and ensuring healthcare professionals act responsibly.
- Particular issues are raised in the context of experimental advanced therapies (such as gene and stem cell therapies), fertility treatment 'add-ons', and innovation in surgery.
- A core challenge is balancing the interests of patients in accessing experimental treatments and the need to support innovation, with ensuring there are sufficient safeguards to protect patients from potential harm(s).

### INTRODUCTION

In some circumstances, patients can access medical treatments before they have been subject to rigorous testing and approved by medical regulators, which are often referred to as "experimental treatments". Patients might access these treatments when they are in a desperate situation, have exhausted all other options, or are

not able or do not want to wait for the approval process.<sup>1</sup>

This briefing note considers why, and in what contexts, patients might access experimental treatments, how they are regulated in the UK, and ethical questions raised by their use.

# BIOETHICS BRIEFING NOTE

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## Disagreements in the care of critically ill children



### OVERVIEW

- Protracted disagreements between parents and healthcare staff about the care and treatment of babies and young children who are critically ill can have damaging effects on everyone involved.
- Law and professional guidance state that decisions about care and treatment must be made in the child's best interests, and that parents and healthcare staff ideally should both agree to the care and treatment that is to be provided. Whether a different approach to decision making should be taken is the subject of current debate.
- The reasons why disagreements develop are wide ranging, but themes include: communication issues; differing perspectives, beliefs and values that lead to disagreements on, for example, what kind of risks justifiably could be taken; feelings of powerlessness for both parents and staff; and delays in seeking resolution interventions.
- The wider social and cultural context is also likely to be playing a role, including the increasing place of social media in public debate, easy access to online information about medical treatments, and the severe financial pressures facing the NHS.
- There is scope for policy makers and others to do more to support the creation of healthcare environments that foster good, collaborative relationships between parents and healthcare staff. The aim should be: good communication between families and staff and an understanding of differing perspectives; appropriate involvement of parents in discussions and decisions about the care and treatment of their child; timely use of effective resolution interventions in cases of disagreement; and attention to the profound psychological effects that disagreements can have on families and staff.

# UK provisions for accessing experimental treatments

Medicines	Medical devices and implants
<ul style="list-style-type: none"><li>• Specials Exemption (EU Compassionate Use)</li><li>• Early Access to Medicines Scheme</li></ul>	<ul style="list-style-type: none"><li>• Exceptional Use</li><li>• In-house Exemption</li></ul>
Advanced therapies	All
<ul style="list-style-type: none"><li>• Hospital exemption</li><li>• Specials exemption</li></ul>	<ul style="list-style-type: none"><li>• Research</li><li>• Off-label prescribing</li><li>• Emergencies</li></ul>

# Equity of access issues

- Not standard practice for NHS to fund experimental treatments
- Variations between regions & countries
- Cancer Drugs Fund
- Sometimes funded by manufacturer
- Crowdfunding – but not always enough

**£1.27 billion Cancer Drugs Fund a political 'quick-fix' that harmed some patients, review finds**

In UK and Ireland, SMA Patients Demand Reimbursement for Spinraza

MARCH 6, 2019 BY LARRY LUXNER IN NEWS



# Challenges for decision making

- Uncertainty about safety and efficacy
- Highly stressful situations
- Doctor-patient dynamics
- Online information and language
- Marketing activities
- Conflicts of interest

News

US makers of joint replacements are fined for paying surgeons to use their devices

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### FDA News Release

**FDA warns US Stem Cell Clinic of significant deviations**

thebmj





## UK law and guidance says....

- Treatment & care decisions should be made in **best interests** of child
- **Parents must consent** to any intervention
- **No obligation on doctors** to provide treatment they do not believe is in child's best interest
- Upshot: Doctors and parents should ideally agree on care and treatment through process of **shared decision making**

### **What happens when they disagree?**

Discussion, second opinions, clinical ethics advice, mediation, court

## Ashya King, 2014

- Brain cancer
- Proton therapy
- NHS decided not to fund
- Parents took Ashya abroad and arrested
- Court ruled for parents
- Ashya has therapy in Prague
- Ashya cleared of cancer

## Charlie Gard, 2017

- Mitochondrial disease
- Nucleoside therapy
- Crowd-funded £1.2M
- Hospital decided not to support trial
- Judges decide in favour of hospital
- Charlie's life support withdrawn



# What's at the root of disagreements between parents and healthcare staff?

1. Communication issues

Conflicting messages, language, break down of trust

2. Differing perspectives

On justifiable risks, on value of life, on who should decide

3. Feelings of powerlessness

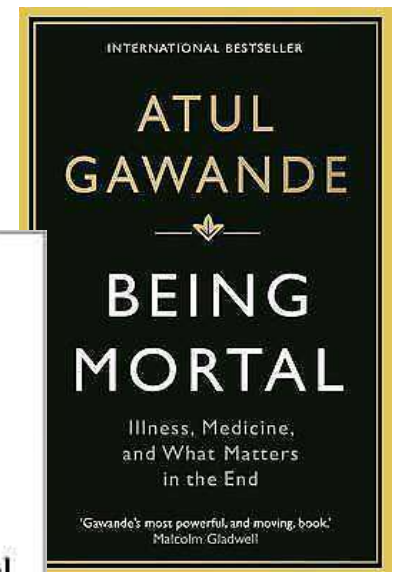
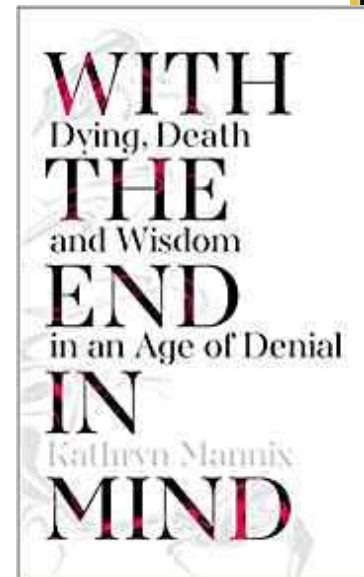
Parents excluded, staff not supported

4. Delays in seeking help

Mediation, clinical ethics committees, palliative care specialists

## Also – the social and cultural context

- Advances in medicine
- Avoidance of talking about death and dying
- Online medical information
- Religious and cultural differences
- NHS financial pressures
- Media and social media



## 'There's nothing to lose': Charlie Gard's mother pleads for US treatment

Parents of critically ill baby lead rally outside Great Ormond Street hospital as they launch fresh legal bid for him to be allowed to travel for treatment



**Charlie Gard case: Latest report 'makes sad reading'**

© 21 July 2017

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Charlie Gard case



## US doctor's intervention in Charlie Gard case 'raises ethical questions'

Critics say Michio Hirano should have been told by judge to see the critically ill baby in person before giving court his opinion



**Terminally ill boy denied 'potentially life-saving' treatment by NHS 'would be given it in any US hospital'**

**Charlie Gard and Alfie Evans cases cost NHS half a million in legal fees after lengthy court battles**

## *Dispute Over British Baby's Fate Draws In Pope and U.S. President*

By [Dan Bilefsky](#) and [Sewell Chan](#)

July 3, 2017

[f](#) [t](#) [e](#) [s](#)

# How can disagreements be avoided or resolved more quickly?

1. Good communication between families and staff
2. Involvement of parents in discussions and decisions
3. Timely use of effective resolution interventions
4. Attention to profound psychological effects on families and staff



Evelina Resolution Project

# Charlie's Law

- Proposes to allow parents to seek '**disease modifying**' treatment if there is a **reputable institution** willing to provide it and it does not cause child **significant harm**
- If a risk of significant harm, a court could be asked to decide what's in the child's best interests
- Analogies with care proceedings
- Threshold of significant harm was rejected in the case of Alfie Evans, 2018

*“not consistent with the founding rule of best interests”*

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