



**Erasmus MC**  
University Medical Center Rotterdam

*Nice to meet? Ethical issues surrounding efforts to facilitate early access to investigational drugs*

**Eline M. Bunnik**

**NEC Forum**  
**The Hague, 11<sup>th</sup> May 2016**



**NWO**  
Nederlandse Organisatie voor Wetenschappelijk Onderzoek



**Responsible Innovation**  
Maatschappelijk Verantwoord Innoveren

**Conditions for expanded access**

- serious/life-threatening disease
- no alternative
- potential for benefit
  
- not eligible for trial participation
  
- approval from drug regulatory authority/inspectorate
- approval from IRB

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Nederlandse Organisatie voor Wetenschappelijk Onderzoek



Research project: 'Nice to meet? Meeting unmet medical needs: a social innovation to facilitate early access to investigational drugs' (2015-2017)

Responsible Innovation (MVI) programme at the Netherlands Organisation for Scientific Research (NWO), to stimulate research on ethical and societal issues surrounding an innovative product or service during the design and development process.

Top sector: Life Sciences & Health



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### Interview study: medical doctors

- Lack of knowledge/experience
- Dissatisfaction with process
- Uncertain outcome
  - inspectorate
  - pharmaceutical company
  - funding

Low uptake in the Netherlands



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## Right to Try movement in USA

The screenshot shows the Goldwater Institute website. The main article is titled "NORTH DAKOTA BECOMES 14TH STATE TO ALLOW TERMINALLY ILL TO ACCESS INVESTIGATIONAL MEDICATIONS". The author is Starlee Coleman. The article text states: "Bismarck—Governor Jack Dalrymple has signed SB 2259—the North Dakota Right To Try Act—into law. The Right to Try Act allows doctors to prescribe medicines to the terminally ill that are being used in clinical trials but are not yet on pharmacy shelves. Right To Try expands access to potentially life-saving treatments years before patients would normally be able to access them. Senator Mathern and Representative Oversen sponsored the bill and led the in-state efforts to pass the law. "We all know the pain of losing someone we love to a terminal illness," said Darcy Olsen, the president of the Goldwater Institute, the group leading the national, bipartisan Right To Try effort. "If you know there's a treatment that is helping people survive, who

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The screenshot shows the myTomorrow's website banner. The headline is "Expanding access to drugs in development". Below it, it says "Start by searching the early access database." There is a search input field with the placeholder text "Enter a medical condition...". The banner features a photograph of a female doctor and an elderly male patient. At the bottom, there are four blue icons representing a globe, two people, a pharmacy, and a bar chart. A "myTomorrow's Team" chat widget is visible in the bottom right corner.

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**myTomorrows**



Find uniform information on medical conditions, related clinical trials and early access programs



Reach out to our in-house team of experts for information



Request access to drugs in development and order diagnostic tests



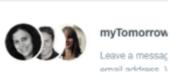
Share treatment data to further improve medical decision-making

79420 **CT**

Clinical trials

172 **EA**

Early access programs



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**From an exemption to a default option?**

“The named patient programme was intended as a valve in the system (...) For that one patient who would otherwise have to wait until the entire system has made sure that [the drug] works for the whole group. (...) That would not be reasonable. (...) But it would be inconvenient if this exception would become the rule.”



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“It is very dangerous to offer false hope to patients who have run out of options, for a fee.” (W. Wind, NPCF, 2013)

mytomorrow

## Our research project

Aim: to develop an ethical framework for a responsible design of efforts to facilitate early access to investigational drugs

Approach:

- Interviews with stakeholders (n = 32) in the Netherlands
- Qualitative (and quantitative) studies of patients' views in the Netherlands, US and Turkey (focus groups and survey)
- Qualitative (and quantitative) studies of physicians' views in the Netherlands, US and Turkey (interviews and survey)

## Acknowledgements

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