



Emma Center for Personalized Medicine Registration form Therapy clinic

Name, function, e-mail, telephone:

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What is the question for the Therapy clinic (e.g. rationale, intervention, methodology, safety, feasibility, outcome measures, regulations, accessibility)?

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Does it concern an (experimental) therapy in care setting or clinical trial?

- (Experimental) therapy in care setting
- Clinical trial
- To be determined

Please complete this form. If any information is unknown, clearly indicate this in each relevant section and, if applicable, include it as a question for the Therapy clinic. Send the completed form to emmacpmtherapie@amsterdamumc.nl:

Case / Cases

How many patients would you like to treat?	
Description of disease and patient(s): <ul style="list-style-type: none"> • Diagnosis (OMIM) • Gene and variant including classification (OMIM) • If metabolic, specify • Phenotype • Treatment goals • Age • If developmental delay, IQ or degree of intellectual disability • Relevant comorbidities 	
Is information available about the natural course of the patient(s)/disease(s)?	

Therapy

Medicine?	<input type="checkbox"/> Yes, novel <input type="checkbox"/> Yes, repurposing (registered product for another indication) <input type="checkbox"/> No
Nutrition (supplement)?	<input type="checkbox"/> Yes, supplement <input type="checkbox"/> Yes, diet <input type="checkbox"/> No
RNA therapy?	<input type="checkbox"/> Yes <input type="checkbox"/> Nee
Advanced Therapy Medicinal Product (ATMP), including gene therapy?	<input type="checkbox"/> Yes <input type="checkbox"/> No

Shortly explain the intervention, including: <ul style="list-style-type: none"> • Mechanism • Expected effect(s) and duration • Safety • Registered product • Availability 	
What is the rationale? Why would this therapy be better compared to current care and treatment?	
Is placebo available? Is a (hospital) pharmacist or pharmaceutical company involved?	

Design

Is there an idea for study design?	<input type="checkbox"/> Yes, namely <input type="checkbox"/> No <input type="checkbox"/> Partially
How many patients can participate?
Is there already a (METC) protocol?	<input type="checkbox"/> Yes <input type="checkbox"/> No <input type="checkbox"/> Partially

Other

What type of trial is it?	<input type="checkbox"/> Investigator-initiated <input type="checkbox"/> Pharma-driven or sponsored <input type="checkbox"/> Other
At what notice are patient(s) able to start?
Is it urgent?	<input type="checkbox"/> Yes <input type="checkbox"/> Somewhat <input type="checkbox"/> No
Are there safety issues?	<input type="checkbox"/> Yes <input type="checkbox"/> No
Is there a trial ongoing elsewhere?	<input type="checkbox"/> Yes <input type="checkbox"/> No (as far as I know)
Who will be the treating physician?	
Outpatient or clinical?	<input type="checkbox"/> Clinical <input type="checkbox"/> Outpatient <input type="checkbox"/> Other / unknown
Are there any other relevant issues to discuss?	
Literature (minimal 2 PMIDs):	