

Application form Research Project CPS

[June 2016]

Coordinator FA-CPS-380: Dr. Monique Slijper; m.slijper@uu.nl; 030-253 3789

Only after official approval by 1) Dr. Monique Slijper, and 2) the Board of Examiners, you can start the Research Project

Please type full text if necessary, the size of all cells should automatically adjust to text sizes

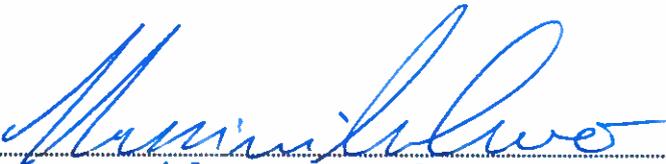
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Which CPS courses did you pass successfully? ²⁾	CPS101, 102, 103, 104, 211, 213, 221, 222
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¹⁾ UU-mail address²⁾ You should have passed CPS101, 102, 103, 104, 211/212/213 and 221/222/223³⁾ Examiner 1 should be employed as staff member, i.e. Professor, Associate professor (UHD) or Assistant professor (UD) at Utrecht University.⁴⁾ Only fill in if daily supervisor is not Examiner 1. The daily supervisor can be a PhD, or post-doctoral researcher at the same group as examiner 1.⁵⁾ Examiner 2 should be employed as staff member, i.e. Professor, Associate professor (UHD) or Assistant professor (UD) at the Department of Pharmaceutical Sciences.

Project Information	
Project title	CRISPR/Cas9 Delivery with synthetic vectors.
Period	From: September 2017 Till: February 2018
Project description (length approx. A4; research topic, main research questions/research goals, short description of experimental setup)	
<p>Niemann-Pick disease is an inherited metabolic disorder characterised by accumulation of sphingomyelin in lysosomes. The disorder arises due to mutations in the SMPD1 gene that encodes acid sphingomyelinase, which breaks down sphingomyelin. These mutations produce a deficiency in the normal activity of the enzyme. Other mutations that can give rise to Niemann-Pick disease include those in NPC1 & NPC2, which normally regulate lipid transport. The disorder is prominent in the spleen, the heart and the brain. Currently there is no effective treatment. Genzyme is currently developing an enzyme replacement therapy, but such treatments are expected to be expensive and require lifelong treatment.</p> <p>The rise of CRISPR/Cas9 genome engineering has brought hope for the treatment of genetic disorders, since it can be tuned to target specific loci with high accuracy and efficiency. The major drawback for the use of Cas9 for in vitro genome engineering is the delivery of this system. Currently a lot of viral vectors, such as adeno-associated virus, have been used, but the possibility of using synthetic factors is also being slowly explored.</p> <p>This project will focus on the delivery of SpCas9 (from <i>Staphylococcus pyogenes</i>) using synthetic factors. For this to be accomplished, first the SpCas9 will be produced from appropriately transformed <i>E. coli</i> cells and will be purified. Upon production and purification, it will be possible to address the main question. This will be addressed by using cell lines (have yet to be determined) and delivering the SpCas9 using different synthetic factors in order to correct the defective gene. Then, the efficiency and accuracy of the targeting will be determined.</p>	

Agreements between student and supervisor	
Absence of student for holiday, courses, training elsewhere, etc.	1) Workshop/assignments obligatory for all FA-CPS-380 CPS students, mostly each last Friday of the month; details of each meeting will follow by e-mail. 2) ... 3) ...
Absence of daily supervisor for holiday, courses, training elsewhere, etc.	
Evaluation moments of student's performance (frequency)	Group meetings and personal meetings
Presentations	1 final presentation
Lab/group meetings	Group meeting every Tuesday 8:30
Other activities to be attended	
Assessment criteria in addition to standard (i.e. practical work, written report, and presentation)	
By signing this document, the student declares to transfer the copyright of any and all products, including the tangible and intellectual products, of the research project to Utrecht University, University Medical Centre or the host Institute. The rights of the student by scientific standards to be a co-author of publications or to be otherwise acknowledged are still recognized.	

General Agreements	
1.	Both examiners give their evaluations independently. The daily supervisor 1 can just advise examiner 1.
2.	Examiner 1 sends the assessment form of the research project to 'Students' administration office', Buys Ballot Building, room 124a, Utrecht
3.	The student provides the final Research Report to 1) Monique Slijper (m.slijper@uu.nl), and 2) to the 'Students' administration office' in pdf-format via e-mail to science.pharm.ba@uu.nl

Signatures of all parties	
Signature of Student:	Student:  <hr/> Date: 19-6-2017
Signature of Examiner 1	Examiner 1  <hr/> Date: 19-6-2017
Signature of Examiner 2	Examiner 2  <hr/> Date: 19-6-2017
Signature of CPS-380 coordinator, Dr. M. Slijper	CPS-380 coordinator:  <hr/> Date: 3 juli 2017
Signature of Board of Examiners	Responsible board member: <hr/> Date: