

# Scientific Programme

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Friday 28<sup>th</sup> September 2018

09:30-10:00	Arrival / Coffee	
10:00-10:10	Welcome	<i>Arjan Lankester</i>
10:10-10:40	Stem cell transplantation in PID/SCID	<i>Alain Fischer</i>
10:40-11:10	Gene therapy in SCID	<i>Fabio Candotti</i>
11:10-11:30	Coffee Break	
11:30-12:00	Lessons from RAG genetics	<i>Luigi Notarangelo</i>
12:00-12:30	Impact of newborn screening	<i>Sung-Yun Pai</i>
12:30-13:30	Lunch	
13:30-14:00	Reduced intensity conditioning	<i>Paul Veys</i>
14:00-14:30	Stem Cell Transplantation in DNA repair disorders	<i>Andrew Gennery</i>
14:30-15:00	Coffee Break	
15:00-15:30	Stem cell transplantation in PID: who benefits?	<i>Stephan Ehl</i>
15:30-16:00	Position of HSCT in adolescents and adults with PID	<i>Emma Morris</i>
16:00-16:30	Restoration of immunity after HSCT in SCID	<i>Arjan Lankester</i>
16:30-17:00	Long term complications and follow-up after HSCT in PID	<i>Benedicte Neven</i>
19:00-20:30	Educational Day Networking Event	



# Saturday 29<sup>th</sup> September 2018

**13:00-14:00 Lunch**

**14:00-15:15 SCID and international NBS experience**

*Chairs: R. Bredius / C. Booth / C. Speckmann*

United Kingdom	<i>A. Gennery</i>
Netherlands	<i>R. Bredius</i>
Germany	<i>C. Speckmann</i>
Sweden	<i>A. Fasth</i>
Spain	<i>O. Neth, A. Martin</i>
South East Asia	<i>K. Imai</i>
United States	<i>M. Cowan, C. Dvorak</i>
Italy	<i>F. Barzaghi</i>

**15:15-15:45 Coffee Break**

**15:45-17:00 Inherited immune disorders: HSCT or not:**

*Chairs: B. Neven / M. Slatter / M. Hönig*

OAS1	<i>F. Hauck</i>
LRBA	<i>M. Seidel, P. Stepensky, S. Bakhtiar</i>
RIPK	<i>S. Hambleton</i>
G6PC3	<i>A. Goenka</i>

# Saturday 29<sup>th</sup> September 2018

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## 17:00-17:45 IEWP Studies: Short (interim) reports and proposals

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*Chairs: A. Lankester / M. Albert*

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Report IEWP-EBMT Leiden office/ SCETIDE office

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CGD

*R. Chiesa, M. Slatter,  
T. Güngör*

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WAS

*M. Albert*

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IFNgR

*P. Olbrich, M. Hönig,  
R. Bredius, S. Holland*

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ICF

*D. Berghuis*

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CD27

*S. Ghosh, A. Lankester*

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Hypomorphic RAG transatlantic study

*B. Neven, C. Schuetz,  
G. Notarangelo*

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## 20:00-24:00 IEWP Social Dinner

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# Sunday 30<sup>th</sup> September 2018

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## 08:30-10:00 Gene therapy in PID and innovative conditioning

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*Chairs: A. Aiuti / A. Lankester*

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GT in WAS: long term follow-up *C. Booth*

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Gene editing in CD40L deficiency *P. Genovese*

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GT in X-SCID *M. Cowan*

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SCIDNET: ADA trial update *C. Booth*

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RECOMB: interim report *F. Staal*

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Conditioning with c-kit antibodies *R. Agarwal*

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## 10:00-10:30 Coffee Break

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## 10:30-11:45 Special challenges and new approaches

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*Chairs: G. Lucchini / K. Kalwak*

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HSCT in patients with lymphoproliferative disorders and primary immunodeficiency *G. Prunotta*

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Rubella associated granulomatous (cutaneous) disease *B. Neven, F. Hauck*

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G-CSF plus plerixafor addition to the conditioning regimen before TCR $\alpha\beta$ + / CD19+ graft depleted HSCT in WAS patients *D. Balashov*

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*Reduced intensity CBT plus CB-DLI in inherited disease: Pittsburgh experience* *M. vander Lugt*

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## 11:45-12:30 Management of TMA

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*Chairs: P. Svec / R. Chiesa*

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Eculizumab in TA-TMA *P. Svec*

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Coversin in TA-TMA *R. Chiesa*

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Defibrotide in TA-TMA *A. Gennery*

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12:30 **Final Remarks** *Arjan Lankester*

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