

## DAY I - Monday, 6<sup>th</sup> May 2019

**09.30-10.30 Registration and coffee**

Atrium | All participants

### Welcome

Main Seminar Room MOA 5 | All participants

**10.30-10.35 Welcome and objectives of the kick-off meeting**

Hans-Dieter Volk, Petra Reinke - Charité - Universitätsmedizin Berlin, Germany

**10.35-10.45 Welcome Address**

Axel Pries - Dean of the Medical Faculty of the Charité and Chairman of the BIH Executive Board (interim)

### Introduction

Main Seminar Room MOA 5 | All participants

**10.45-11.20 RESTORE - Health by Advanced Therapies - Why, what, how, who?**

Hans-Dieter Volk - Charité - Universitätsmedizin Berlin, Germany (20 min presentation + 15 min panel discussion)

**11.20-11.50 Introduction of RESTORE core team**

Charité/BIH, Univ Zurich, Catapult, TissUse, Pluristem, Miltenyi Biotech, Inserm/Univ Paris Saclay, InnActa, Telethon, Univ. Minho  
(3 min/each partner, total 30 min)

**11.50-12.15 Introduction into roadmap and working groups**

Hans-Dieter Volk - Charité - Universitätsmedizin Berlin, Germany (20 min)

### Break

Atrium | All participants

**12.15-13.10 Lunch**

### I. Overarching Elements

Main Seminar Room MOA 5 | All participants

**13.15-13.40 Ethics**

**13.40-14.10 Big Data / Artificial Intelligence to handle product characteristics, biomarker, (pre)clinical outcome**

Lead: Pluristem, Co-Lead: Catapult

**14.10-14.30 Economic aspects: Health technology assessment, reimbursement**

## II. RIA Module "Research Platform Manufacturing" - Working groups 1-3

Main Seminar Room MOA 5 | All participants

**14.30-15.00 WG 1: Manufacturing: somatic and gene-modified cells**

Lead: Pluristem, Co-Lead: Miltenyi, Inserm

**15.00-15.30 WG 2: Manufacturing: tissue engineering & composite products**

Lead: University Zurich, Co-Lead: University Minho

**15.30-16.00 WG 3: Manufacturing: *in vivo* gene therapy and gene editing**

Lead: Telethon, Co-Lead: Charité

### Coffee break

Atrium | All participants

16.00-16.20 *Coffee break*

### Break-out sessions (WG 1+2+3 in parallel):

Seminar Room MOA 3 | Interested participants

**16.25-17.00 WG 1: Manufacturing: somatic and gene-modified cells (30 min)**

Seminar Room MOA 4 | Interested participants

**16.25-17.00 WG 2: Manufacturing: tissue engineering & composite products (30 min)**

Seminar Room MOA 1 | Interested participants

**16.25-17.00 WG 3: Manufacturing: *in vivo* gene therapy and editing (30 min)**

## III. RIA Module "Research Platform Manufacturing" - Working groups 4-5

Main Seminar Room MOA 5 | All participants

**17.00-17.30 WG 4: Manufacturing: pluripotent stem cells and adult stem cells**

Lead: Inserm, Co-Lead: Telethon

**17.30-18.00 WG 5: Manufacturing: *ex vivo* gene delivery / gene editing**

Lead: Catapult, Co-Lead: Miltenyi

### Break-out sessions (WG 4+5 in parallel):

Seminar Room MOA 3 | Interested participants

**18.10-18.40 WG 4: Manufacturing: pluripotent stem cells and adult stem cells (30 min)**

Seminar Room MOA 4 | Interested participants

**18.10-18.40 WG 5: Manufacturing: *ex vivo* gene delivery / gene editing (30 min)**

## Sum-up day I

Main Seminar Room MOA 5 | All participants

18.45-19.15

### Summary break-out sessions day I

Leaders of break-out sessions WG 1-5

19.15-19.20

### Closing remarks day I

Hans-Dieter Volk - Charité - Universitätsmedizin Berlin, Germany (5 min)

## Networking get-together

Atrium | All participants

19.20-20.45

*Cheese & Wine*

## DAY II - Tuesday, 7<sup>th</sup> May 2019

### 08.00-08.25 Registration day II

Atrium | All participants

#### Welcome

Main Seminar Room MOA 5 | All participants

### 08.25-08.30 Welcome day 2

Hans-Dieter Volk - Charité - Universitätsmedizin Berlin, Germany (5 min)

#### IV. RIA Modules "Preclinical Models" and "New Clinical Applications" - Working groups 6-9

Main Seminar Room MOA 5 | All participants

### 08.30-09.00 WG 6: Pre-clinical model systems: *in vitro* and *in vivo*

Lead: TissUse, Co-Lead: Inserm

### 09.00-09.30 WG 7: New clinical applications: support of endogenous regeneration

Lead: Charité, Co-Lead: Pluristem

### 09.30-10.00 WG 8: New clinical applications: tissue and organ replacement

Lead: Telethon

Co-Lead: University Zurich

### 10.00-10.30 WG 9: New clinical applications: cancer

Lead: Miltenyi, Co-Lead: Inserm

#### Coffee break

Atrium | All participants

10.30-10.55 *Coffee break*

#### Break-out sessions (WG 6+7 and 8+9 in parallel):

Seminar Room MOA 3 | Interested participants

11.00-11.30 **WG 6: Pre-clinical model systems: *in vitro* and *in vivo*** (30 min)

Seminar Room MOA 4 | Interested participants

11.00-11.30 **WG 7: New clinical applications: support of endogenous regeneration** (30 min)

Seminar Room MOA 3 | Interested participants

11.35-12.05 **WG 8: New clinical applications: tissue and organ replacement** (30 min)

Seminar Room MOA 4 | Interested participants

11.35-12.05 **WG 9: New clinical applications: cancer** (30 min)

## V. RIA Modules "Regulatory Science & Health-Technology Assessment", "Early Clinical Trials" and "Late Clinical Trials" - Working groups 10-12

Main Seminar Room MOA 5 | All participants

**12.10-12.40** **WG 10: Clinical research: regulatory science, refined translation, early HTA & early clinical trials**  
Lead: Charité, Co-Lead: Catapult

**12.40-13.10** **WG 11: Clinical research: pivotal clinical trials and marketing authorization**  
Lead: Telethon, Co-Lead: Pluristem

**13.10-13.40** **WG 12: Post-Trial and long-term follow-up, data warehouse and patient registry**  
Lead: Telethon, Co-lead: Pluristem

### Break

Atrium | All participants

**13.40-14.35** *Lunch*

### Break-out sessions (WG 10+11 in parallel, WG 12):

Seminar Room MOA 3 | Interested participants

**14.40-15.10** **WG 10: Clinical research: regulatory science, refined translation, early HTA & early clinical trials (30 min)**

Seminar Room MOA 4 | Interested participants

**14.40-15.10** **WG 11: Clinical research: pivotal clinical trials and marketing authorization (30 min)**

Seminar Room MOA 3 | Interested participants

**15.15-15.45** **WG 12: Post-Trial and long-term follow-up, data warehouse and patient registry (30 min)**

## VI. RIA Module "Implementation & Exploitation" - Working Groups 13-14

Main Seminar Room MOA 5 | All participants

**15.50-16.20** **WG 14: Valuation and innovative reimbursement models for new Advanced Therapies**  
Lead: Charité, Co-lead: Catapult

**16.20-16.50** **WG 13: Implementation of new Advanced Therapies into clinical routine**  
Lead: Miltenyi, Co-lead: Telethon

### Coffee break

Atrium | All participants

**16.50-17.10** *Coffee break*

## Summary break-out sessions day II

Main Seminar Room MOA 5 | All participants

17.15-18.00

### Summary break-out sessions day 2

Leaders of break-out sessions WGs 6-12 (5 min each, total 35 min)

## Next Steps

Main Seminar Room MOA 5 | All participants

18.00-18.15

### Dissemination during preparatory phase

Charité - Universitätsmedizin Berlin, Germany (15 min)

18.15-18.20

### Science meeting Nov 2019 and patient advocacy group meeting(s)

Hans-Dieter Volk, Petra Reinke - Charité - Universitätsmedizin Berlin, Germany (5 min)

18.20-18.25

### Closing remarks and farewell

Hans-Dieter Volk - Charité - Universitätsmedizin Berlin, Germany (5 min)

18.30

### End of day II

## End of programme