

# EB2017 Research & EB-CLINET Conferences 2017

**24-27 September 2017**

Crowne Plaza Salzburg – The Pitter  
Rainerstrasse 6-8, Salzburg

**Two conferences,  
one shared day**



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# Welcome to Salzburg!

We are delighted to welcome the global EB research and clinical communities back to Austria in 2017, to review the progress made in research and clinical practice to help people living with epidermolysis bullosa. DEBRA Austria previously hosted DEBRA International's research conference in Vienna 2009. This time, we are pleased to welcome you all to Salzburg, a beautiful city and also home to DEBRA Austria's 'EB House'.

Celebrating its 12<sup>th</sup> anniversary this year, EB House Austria is the first initiative of its kind worldwide, providing an integrated centre of EB clinical expertise, training and research. This focus on support for research and emphasis on the need for its ultimate clinical application, is at the core of DEBRA's goals to improve daily quality of life for all people with EB, while also seeking eventual cures and better treatments targeting the underlying causes of EB.

The goal of research for patient benefit also underpins our decision to bring together, for the first time, DEBRA International's research conference, EB2017, and the meeting of the international network of EB clinical centres and experts, EB-CLINET. We are delighted to see that so many delegates have chosen to attend both conferences, as a greater mutual understanding of the challenges faced by the research and clinical communities will speed up progress. Appreciating the daily clinical challenges of managing life with EB is a prerequisite to formulating appropriate clinical-trial protocols, and designing practical treatments that address patient priorities.

We encourage you to make the most of the conferences – for everyone's benefit – by sharing your knowledge and experience, asking questions and raising issues you feel are important. At both the conference formal sessions and social events, please do introduce yourself and your work to people you have not met before! 

We wish you successful conferences and hope you enjoy your time in Salzburg!



**Dr Rainer Riedl**

President, DEBRA Austria

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## Welcome to DEBRA International's Research Conference EB2017

EB2017 marks three decades of research support in DEBRA's mission to improve life options for people with EB. The past decade has seen the introduction of diverse new approaches to treating EB, and many of these have made a rapid transition from therapeutic concept to clinical trials. Exciting new technologies potentially offer curative treatments, but can also pose challenges in implementation, both technological and regulatory. Improved understanding of EB reveals new therapeutic targets; molecular messengers in chronic inflammation play roles in problems as diverse as itch, poor wound healing and the devastation of cancer in severe EB. An integrated picture of EB will lead to better integrated care. A key theme of EB2017 will be to review what we have learned over the past decade, and how this will guide what we do next. Sharing of failures as well as successes will help overcome barriers to clinical translation, as well as to prioritize future DEBRA funding. We are pleased to welcome representatives from biopharma and healthcare companies: partnership is essential to delivering better health to people with EB. At this conference we are also giving more speaking opportunities to younger researchers to present their research – we welcome the enthusiasm and ideas of the 'next generation' of leading EB researchers! 🌸

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**Dr Clare Robinson**

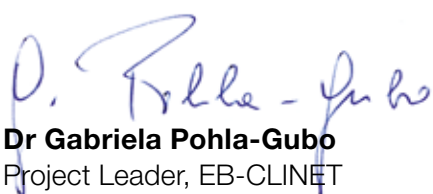
Head of Research, DEBRA International



## Welcome to the 4<sup>th</sup> Conference of EB-CLINET

A warm welcome to the 4<sup>th</sup> Conference of EB-CLINET, the international network whose goal is to forge strong clinical partnerships, to share and consequently enhance professional expertise, and to optimise clinical care for people with epidermolysis bullosa (EB). We're delighted to share one day with this year's conference on EB Research, EB2017, in recognition of the evolution of preclinical research into a growing number of clinical trials – ,from lab-bench to bedside'. Naturally, addressing hot topics in EB care will remain the key focus in dedicated EB-CLINET sessions. Moreover, we will also debate broader issues such as ,how to evaluate success in EB treatment' or the role of patients and DEBRA in terms of their involvement in basic research, clinical trials and care. To focus in more detail on particular specialist topics, there are multiple side meetings devoted, for example, to the development of priority clinical practice guidelines. In addition, the conference will highlight the ways in which collaboration with umbrella organisations such as the European Reference Network for Skin Diseases (ERN-Skin) will facilitate patient access to diagnoses and novel treatments through the establishment of an international EB registry. Our joint efforts have the potential to pave the way for further research and new treatment options for the benefit of those living with EB. 🌸

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**Dr Gabriela Pohla-Gubo**

Project Leader, EB-CLINET







# Conference Programme

## Sunday, 24 September | EB2017 only

Time	min	Sunday, 24 September 2017   EB2017 only	Speaker
14:00 – 17:00		<b>Registration for delegates</b>	
17:00 – 17:15	15	<b>Welcome EB2017</b> Rainer Riedl (President of DEBRA Austria) Sabine Gabath (Representative of the City of Salzburg) Mike Jaega (President of DEBRA International)	
<b>Session 1</b> 17:15 – 19:00		<b>Preclinical Therapy Development I</b>	<b>Chairs:</b> L Bruckner-Tuderman / A Hovnanian
17:15 – 17:40	25	<b>Position talk: Challenges and prospects for iPSC + Progress in iPSC for EBS DM</b>	D Roop
17:40 – 18:00	20	Combined creation and correction of iPSC for RDEB	M Wernig
18:00 – 18:20	20	Gene editing in iPSC for DDEB	J Jackow
18:20 – 18:40	20	High-efficiency RNA-based reprogramming of human EB somatic cells	G Bilusova
18:40 – 19:00	20	Discussion	
19:00 – 21:00		<b>Welcome Reception – Foyer, Conference Venue</b>	

# Monday, 25 September | EB2017 only

Time	min	Monday, 25 September 2017   EB2017 only	Speaker
07:15–08:45		Breakfast Meeting (Industry Partnering Panel, by invitation only)	
<b>08:00–09:00</b>		<b>Registration for delegates</b>	
09:00–09:10	10	<b>Tribute to Robin Eady</b>	J-D Fine
<b>Session 2 09:10–11:00</b>		<b>Preclinical Therapy Development II</b>	<b>Chairs:</b> A South / G Meneguzzi
09:10–09:30	20	Genegraft for RDEB	A Hovnanian
09:30–09:50	20	Gene editing for RDEB	F Larcher
09:50–10:10	20	Gene therapy for RDEB	J Bauer
10:10–10:30	20	Addressing immune responses to gene-cell therapy in EB	I Gratz
10:30–10:40	10	Short talk selected from poster: HSV-1 mediated gene therapy for RDEB	I Fuentes
10:40–11:00	20	Discussion	
<b>11:00–11:30</b>	<b>30</b>	<b>Coffee</b>	
<b>Session 3 11:30–13:00</b>		<b>Preclinical Therapy Development III</b>	<b>Chairs:</b> J Bauer / C Niessen (TBC)
11:30–11:50	20	Protein therapy for DEB	P Marinkovich
11:50–12:10	20	Emerging targets and treatment options for EBS	T Magin
12:10–12:20	10	Direct delivery – a gentle polymer for the skin	L Cutlar
12:20–12:30	10	Short talk selected from poster: Low-dose Calcipotriol improves wound healing in EB	C Gruber
12:30–12:40	10	Short talk selected from poster: C7 correction by RNA trans-splicing	P Peking
12:40–13:00	20	Discussion	
<b>13:00–14:00</b>	<b>60</b>	<b>Lunch</b>	
<b>Session 4 14:00–16:00</b>		<b>Inflammation, Fibrosis and Cancer</b>	<b>Chairs:</b> P Marinkovich / G Zambruno
14:00–14:25	25	<b>Position talk: Tissue microenvironment as driver of disease and target for SCC therapy in RDEB</b>	J Marshall
14:25–14:45	20	TGF beta and angiogenesis	E O'Toole
14:45–15:05	20	Inflammation driven mutation acquisition in EB SCC	A South
15:05–15:20	15	A novel disease-modifying treatment for RDEB: a preclinical study targeting the anti-fibrotic AT2 and MAS receptors	A Nystroem
15:20–15:35	15	HMGB1-derived peptide drug to reduce inflammation and fibrosis	K Tamai
15:35–16:00	25	Discussion	
<b>16:00–16:30</b>	<b>30</b>	<b>Coffee</b>	
<b>Session 5 16:30–18:00</b>		<b>Preclinical Therapy Development IV</b>	<b>Chairs:</b> J Uitto / D Murrell
16:30–16:50	20	siRNA therapies for EB, development and effective delivery	R Hickerson
16:50–17:10	20	Use of antisense oligonucleotides to correct RDEB	M Pasmooij
17:10–17:25	15	PTC readthrough opportunities for RDEB therapy: novel candidate drugs	M Roberge
17:25–17:35	10	Short talk selected from poster: Feasibility of aminoglycoside therapy in selected RDEB and H-JEB	M Chen
17:35–17:45	10	Short talk selected from poster: Amlexanox: novel read-through approach for RDEB therapy	V Atanasova
17:40–18:00	15	Discussion	
<b>17:00–19:00</b>		<b>Registration for delegates</b>	
<b>19:00</b>		<b>Traditional Dinner – Pitterkeller, Conference Venue</b>	

# Tuesday, 26 September | EB2017 & EB-CLINET

Time	min	Tuesday, 26 September 2017   JOINT SESSIONS EB2017 & EB-CLINET	Speaker
07:15 – 08:45		Breakfast Meeting (New genotypes, new phenotypes, by invitation only)	
<b>08:00 – 09:00</b>		<b>Registration for delegates</b>	
09:00 – 09:05	5	<b>Welcome EB-CLINET</b>	G Pohla-Gubo
<b>Session 6 09:05 – 10:50</b>		<b>Clinical trials: Gene-cell Therapies – Outcomes</b>	<b>Chairs:</b> A Lane / J Marshall
09:05 – 09:30	25	<b>Position talk: 10 years of gene therapy for EB: what have we learned? (+ a decade of gene therapy development for JEB)</b>	M De Luca
09:30 – 09:50	20	Keratinocyte grafts for RDEB	P Marinkovich
09:50 – 10:10	20	Cell therapies for RDEB: what have we learned?	J McGrath
10:10 – 10:30	20	BMT for RDEB and JEB: what have we learned, and how does this instruct what we do next?	J Tolar
10:30 – 10:50	20	Panel Discussion: Perspectives in Cell Therapies	
<b>10:50 – 11:20</b>	<b>30</b>	<b>Coffee</b>	
<b>Session 7 11:20 – 13:00</b>		<b>Clinical Trials: Symptom-relief Treatments</b>	<b>Chairs:</b> A Heagerty (TBC) / D Kiritsi
11:20 – 11:45	25	<b>Position talk: The trials of organizing a clinical trial for symptom relief (+ antifibrotics trials)</b>	L Bruckner-Tuderman
11:45 – 12:05	20	Topical anti-inflammatory agents for EB	D Murrell
12:05 – 12:20	15	Clinical trial of Oleogel topical treatment for EB	J Kern
12:20 – 12:35	15	Diacerein cream: basal pharmacology and clinical application	V Wally
12:35 – 12:50	15	Panel Discussion: Perspectives in Symptom relief	
<b>12:50 – 13:00</b>	<b>10</b>	<b>Group Picture</b>	
<b>13:00 – 14:00</b>	<b>60</b>	<b>Lunch</b>	
<b>Session 8 14:00 – 15:15</b>		<b>Miscellaneous</b>	<b>Chairs:</b> R Riedl / J Mellerio
		<b>Patient advocacy</b>	
14:00 – 14:15	15	Patient involvement in research: who needs it?	M Jaega
		<b>New EB Mutations &amp; Diagnostic Technologies</b>	
14:15 – 14:30	15	New technologies for improving diagnostic accuracy in EB (+ summary of new mutations)	J Uitto
		<b>Cancer therapy options</b>	
14:30 – 14:45	15	Nivolumab as a putative therapy option in SCC	M Laimer
14:45 – 15:00	15	Life-extending treatment with Cetuximab for metastasized cutaneous SCC in DEB	D Kiritsi
15:00 – 15:15	15	Discussion	
<b>15:15 – 15:45</b>	<b>30</b>	<b>Coffee</b>	
<b>Session 9 15:45 – 17:00</b>		<b>Defining outcomes</b>	<b>Chairs:</b> A Lucky / H Hintner
15:45 – 16:05	20	<b>Position talk: How to evaluate “success” in EB treatment</b>	H Hintner
16:05 – 16:20	15	Natural history of EB – News from PEBLES	J Mellerio
16:20 – 16:35	15	Natural History of Wounds in Patients with RDEB	D Solis
16:35 – 16:50	15	Different subtypes – different outcomes	A Schwieger-Briehl
16:50 – 17:00	10	Discussion	
<b>19:00</b>		<b>Guided Tour &amp; Dinner – Fortress Hohensalzburg</b>	

# Wednesday, 27 September | EB-CLINET only

Time	min	Wednesday, 27 September 2017   EB-CLINET only	Speaker
<b>Session 10</b> <b>09:30–11:00</b>		<b>Hot topics in EB Care I</b>	<b>Chairs:</b> A Martinez / A Diem
09:30–09:50	20	<b>Position talk: From basic research to clinical trials and EB care – what DEBRA can do for EB patients</b>	R Riedl
09:50–10:05	15	Osteoporosis / osteopenia in RDEB – what's new?	A Martinez
10:05–10:20	15	Neuropathic pain	M Calvo
10:20–10:35	15	Hand surgery and rehab	R Cornwall
10:35–11:00	25	Discussion	
<b>11:00–11:30</b>	<b>30</b>	<b>Coffee</b>	
<b>Session 11</b> <b>11:30–13:15</b>		<b>Hot topics in EB Care II</b>	<b>Chairs:</b> F Palisson / E Pope
11:30–11:45	15	Stories from the guideline development panels	K Mayre-Chilton
11:45–12:00	15	Nutrition and the CPG – what's new?	L Hubbard
12:00–12:15	15	Oesophageal strictures	E Pope
12:15–12:30	15	Anaemia	A Lucky
12:30–12:45	15	Prevalence and Characterisation of Cannabis-use to Treat Pain and Pruritus in Epidermolysis Bullosa	N Schröder
12:45–13:00	15	Cannabis in EB – Risks and Potential	A Diem
13:00–13:15	15	Discussion	
<b>13:15–14:15</b>	<b>60</b>	<b>Lunch</b>	
<b>Session 12</b> <b>14:15–15:45</b>		<b>Hot topics in EB Care III</b>	<b>Chairs:</b> A Schwieger-Briehl (TBC) / F Palisson
14:15–14:30	15	Top-line Results From the Phase 3, Double-Blind, Vehicle-Controlled Trial of the Topical Investigational Drug SD-101 in Patients With Epidermolysis Bullosa (ESSENCE study)	A Paller
14:30–14:45	15	Co-occurrence of EB and Marfan syndrome	C Fiorentini
14:45–15:00	15	Wound care as a source of stress	P de Graaf
15:00–15:15	15	EB Clinical Diagnostic Matrix	V Yenamandra
15:15–15:30	15	Discussion	
		<b>Closing remarks</b>	
15:30–15:45	15	EB-CLINET: Connection   Communication   Collaboration	G Pohla-Gubo
<b>15:45</b>		<b>Coffee &amp; Farewell</b>	





# Parallel Sessions (Closed Meetings)

## Breakfast Meetings

Day	Time	Topic	Room
Monday, 25.09.	07:15–08:45	Industry Partnering Panel	Auersperg 1
Tuesday, 26.09.	07:15–08:45	New EB Mutations and diagnostic technologies	Auersperg 2

## CPG Panel Meetings

Day	Time	Topic	Chair	Room
Sunday, 24.09.	14:00–17:00	Diagnostics	Cristina Has	Markus
Monday, 25.09.	09:30–13:30	Anaemia	Irene Lara-Correlas	Markus
Monday, 25.09.	14:00–16:00	Diagnostics	Cristina Has	Markus
Monday, 25.09.	09:30–16:00	Psychosocial Care	Kattya Mayre-Chilton	Auersperg 2
Monday, 25.09.	09:30–16:00	Physiotherapy	Amy Weissman	Sittikus
Tuesday, 26.09.	09:30–16:00	Hand Surgery and Rehab	Roger Cornwall & Rachel Box	Auersperg 1
Tuesday, 26.09.	09:30–16:00	Occupational Therapy	Jennifer Chan	Auersperg 2

## Expert Panel Meetings

Day	Time	Topic	Chair	Room
Monday, 25.09.	14:00–16:00	Hand Surgery	Roger Cornwall	Auersperg 1
Monday, 25.09.	16:00–18:00	Registry Meeting	Fiona Browne	Auersperg 2
Wednesday, 27.09.	13:30–15:00	ERN Meeting (please take lunch into room)	Marcel Jonkman / Jemima Mellerio	Sittikus



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## Evening Events

Please note: Socials must have been pre-booked.  
Please enquire at the registration desk for last minute availability.

### 24 September 2017- 19:00-21:00 | Conference Venue

**Welcome Reception:** Drinks & fingerfood. Kristall Foyer, Hotel Crowne Plaza Salzburg–The Pitter, Rainerstrasse 6-8, 5020 Salzburg

### 25 September 2017- 19:00 | Restaurant Pitterkeller

**Dinner:** Beer tasting followed by a three course evening meal at local traditional Salzburg restaurant. Pitterkeller, Hotel Crowne Plaza Salzburg–The Pitter, Rainerstrasse 6-8, 5020 Salzburg

### 26 September 2017- 19:00 | Salzburg Fortress

**19:00–Guided tour:** Plunge deeper into the fascinating world of the castle  
**20:00–Dinner:** Three course evening meal at Salzburg Fortress high above the baroque towers of the city. Festung Hohensalzburg, Mönchsberg 34, 5020 Salzburg. *Note: you can reach the fortress by walking or via the funicular railway (operates until 23:00)*

Not booked onto a social? Get some information for restaurants, bars or other recommendable places to visit at the registration desk.



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## In Memoriam - Prof Robin Eady

EB2017 is the 5<sup>th</sup> research conference organized by DEBRA International since the EB\*\*\*\* named series of DEBRA research conferences was coined by the late Robin Eady, regarded by many as one of the founding fathers of modern EB research.

Known and appreciated by patients, clinicians and researchers alike, young and old, Robin has been a major source of inspiration and generous with his time and help to all with an interest in EB, including DEBRAs around the world.

Robin Eady 1940 – 2017

We dedicate this conference to his memory.



## EB-CLINET - Linking clinical expertise in EB

Closely linked and encircling the globe, EB-CLINET partners all have a common mission: to further develop specialised medical care for people with EB and to ensure that they have the best possible quality of life.

A rare disease like EB requires the pooling of expertise and experience. EB-CLINET, the Clinical Network of EB Centres and Experts, aims at:

- strengthening the collaboration between medical institutions world wide already providing specialised medical care for people with EB;
- finding „partner centres“ for health care in the field of EB, both within and beyond Europe, and building up EB-relevant knowledge within such centres of EB expertise; and
- setting up an online community and an online database for best practice medical care in EB.

Please visit [www.eb-clinet.org](http://www.eb-clinet.org) for details!



# Welcome to Salzburg

Whatever you're in the mood for, whatever the time of year, every year Salzburg – the city on the Salzach River – attracts countless domestic and foreign guests to a whole range of artistic and cultural events. In Salzburg you'll be spoilt for choice – every day, every evening, every night.

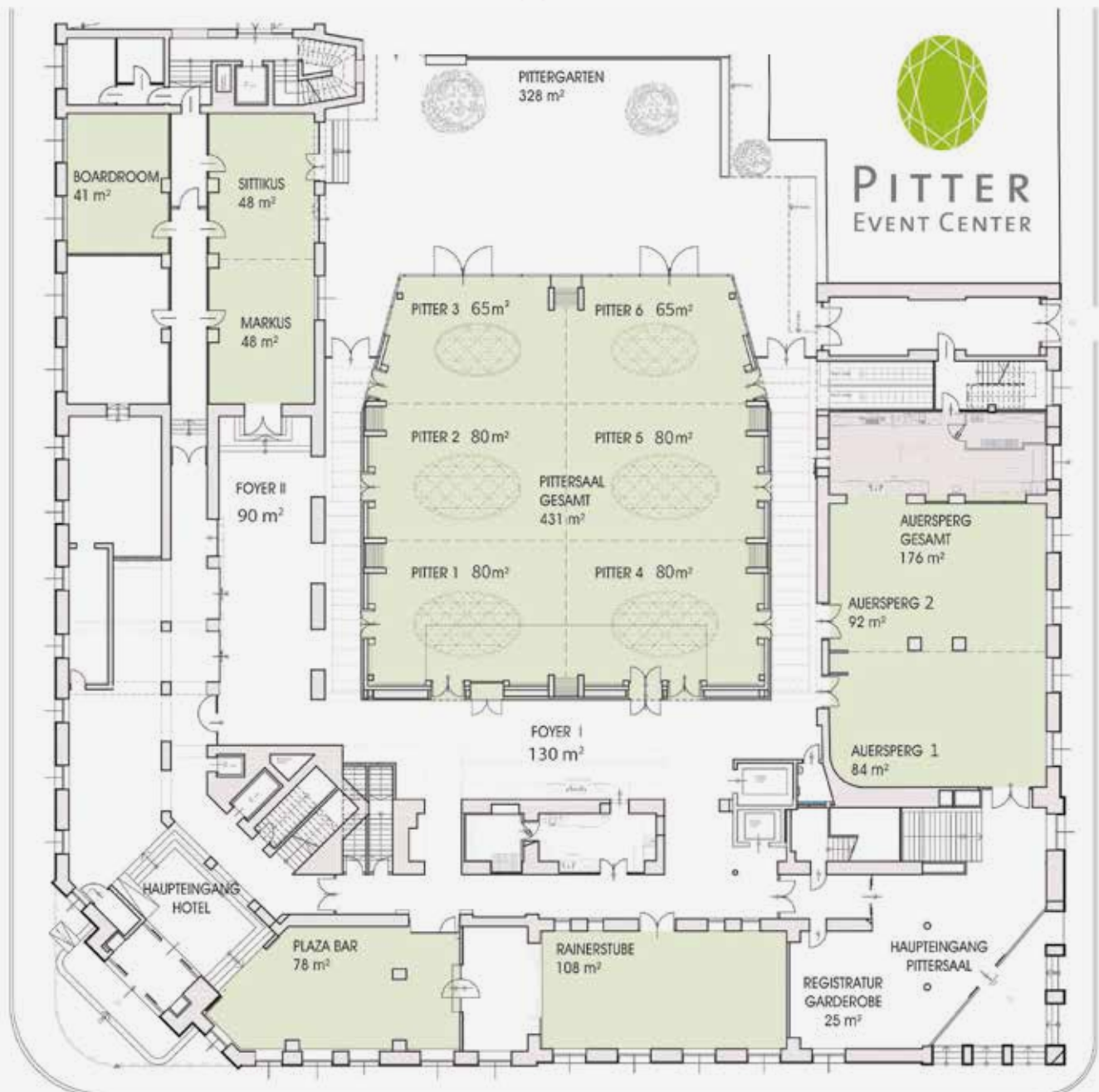
For more information about the city of Salzburg, please visit the official tourist information website: [www.salzburg.info](http://www.salzburg.info)

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## The Venue

  
**CROWNE PLAZA**  
SALZBURG - THE PITTER







### Internet

There will be free wireless internet access at the conference venue.

Name: Pitter Event Center

Password: available at the registration desk

### Lost property

If you lose something please report to main reception desk for assistance.

Found items should be returned to this desk

### Smoking

In the hotel Crowne Plaza–The Pitter operates a strict non-smoking policy.

Please refrain from smoking anywhere inside the venue. Smoking is possible directly outside the hotel doors.

### Disabled access

Next to two barrier-free guest rooms there is a disabled access to all meeting rooms in the conference venue.

### In the event of fire

In case of a fire alarm please leave the building in an orderly fashion by the nearest exit. All fire evacuation routes are clearly marked. Do not use lifts. If you are unable to use the stairs seek assistance from staff or fire marshals or go to the nearest fudge point. If you require the emergency services (fire, police or ambulance) call 112.

### Photographs, videos and audio recordings

Photographs, videos and audio recordings may be taken at this event. These may be used by DEBRA Austria, DEBRA International or EB-CLINET, to record and share the event, promote the work of these organisations and raise awareness of EB. If you have any queries or would like to “opt out” please visit the registration desk.

### Street maps and leisure activities

For street maps and recommendations about leisure activities please check with our main reception desk staff.

### Getting here & parking situation

#### Arrival by car:

The Mirabell-Congress-Garage is located only 2–3 minutes foot walk away from our IMLAUER hotels. It offers 660 parking lots.

Operator: CONTIPARK, Tel: +43 (0) 662 849758-34

- Rate for every 20 min. € 0,90
- Rate from 4 hours € 3,00/per hour
- Rate for 24 hours € 18,00
- Rate after 06:00pm € 5,00/for 12 hours

#### Arrival by public transport:

Salzburg main station is only 7 minutes foot walk away from us.

The total distance is only 650 meters.

The public bus lines 1, 2, 3, 5 and 6 stop at “Kongresshaus” which is located directly in front of the Crowne Plaza Salzburg–The Pitter.



## Premium Plus Sponsors



Castle Creek Pharma is a fully integrated pharmaceutical company with a robust and diversified late stage pipeline of innovative and high potential products in the specialty Dermatology and ENT space as well as other areas of high medical need. We pride ourselves on the strong relationships that we have forged with our key partners in industry, academia and patient based organizations, which have been instrumental in helping us to develop our products and to better address patient needs.



Amryt Pharma is a global biopharmaceutical company dedicated to developing and commercialising innovative therapies that transform the lives of patients with rare, debilitating diseases.

Amryt's has initiated a global phase III study (EASE) to investigate Oleogel-S10 as potential topical wound care treatment for patients with junctional, dystrophic & simplex EB.



Amicus Therapeutics is a global biotechnology company at the forefront of therapies for rare and orphan diseases. Amicus' lead programs in development include migalastat for Fabry disease, SD-101 for Epidermolysis Bullosa, as well as novel enzyme replacement therapy and biologic products for Fabry disease, Pompe disease, and other rare diseases.

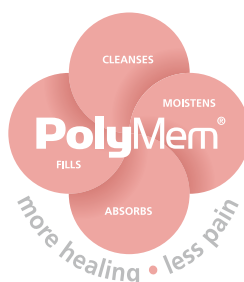


Based in Parma, Italy, Chiesi Farmaceutici is an international research-focused Healthcare Group, with over 80 years of experience in the pharmaceutical industry, present in 26 countries. Chiesi researches, develops and markets innovative drugs in the respiratory therapeutics, specialist medicine and rare disease areas. Its R&D organisation is headquartered in Parma (Italy), and integrated with 6 other key R&D groups in France, the USA, the UK, Sweden and Denmark to advance Chiesi's pre-clinical, clinical and registration programmes. Chiesi employs nearly 5,000 people.



Thank you to CAP Partner for managing the registration and abstract submission for the conferences free of charge and for their ongoing support to DEBRA International and people living with EB

## Premium Sponsors



## Other Sponsors



**THANK YOU to all delegates for attending the EB2017 Research and EB-CLINET conferences**

