Post EBMT - Autoimmunerkrankungen

Prof. Dr. Jörg Henes

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BCMA CAR T-Cell Therapy Demonstrates Early Safety and Efficacy Signals in Refractory Systemic Sclerosis and Antiphospholipid Syndrome: Phase I Results in Autoimmune Diseases

Shlomo Elias[1], Shlomit Kfir-Erenfeld[1], Hadass Pri Chen[2], Nathalie Asherie[1], Bohbot Emilie[3], Rim Elmikawy[3], Shaheen Alaa[3], Ahmad Bazbaz[3], Isam Hendy[3], Yaakov Naparstek[3], Sigal Grisariu[1], Batia Avni[1], Eran Zimran[1], Miri Assayag[1], Nomi Zalcman[1], Alaa Shehadeh[1], Aseel Ishtay[1], Shelly Regev[1], Miriam Schlossberg[1], Elina Zorde-Khevalevsky[1], Elena Karger[1], Marina Krizhevskaya[1], Hagit Peleg[3], Dror Mevorach[3], Chamutal Gur[3‡], Polina Stepensky[1‡]

[1] Department of Bone Marrow Transplantation and Cancer Immunotherapy, Hadassah Medical Center, Faculty of Medicine, Hebrew University of Jerusalem, Jerusalem, Israel.

[2] Department of Nephrology and Hypertension, Hadassah Medical Center, Faculty of Medicine, Hebrew University of Jerusalem, Israel.

[3] Department of Rheumatology, Hadassah medical center, Faculty of medicine, Hebrew University of Jerusalem, Israel.

[‡] These authors contributed equally

Zu Systemischem Lupus existieren schon sehr guter Daten zur Effektivität von Anti-CD19-CAR T Zellen
Single Center Phase I Studie
8 Patienten mit SLE, SSc oder Myositis
Lymphodepletion mit Flu/CY

	Pts 1	Pts 2	Pts 3	Pts 4	Pts 5	Pts 6	Pts 8	Pts 10
Diagnosis	IIM	SSc	SLE	SSc	SLE	SSc	SLE	SLE
Dose	Low	Low	Low	Low	Low	High	Low	High
CRS	None	None	Grade 1	Grade 2	Grade 1	Grade 2	Grade 2	Grade 2
Time to CRS onset (days)	-	-	1	1	3	1	2	0
CRS durations (days)	-	-	3	4	3	5	5	4
Num. Tocilizumab for CRS	-	-	1	2	1	2	3	1
Steroids	-	-	None	None	None	None	Yes	None
Vasopressor use	-	-	None	None	None	None	None	None
ICANS, Grade 1-5	None	None	None	None	None	None	None	None
Other neurological AE's of Grade ≥ 3	None	None	None	None	None	None	None	None

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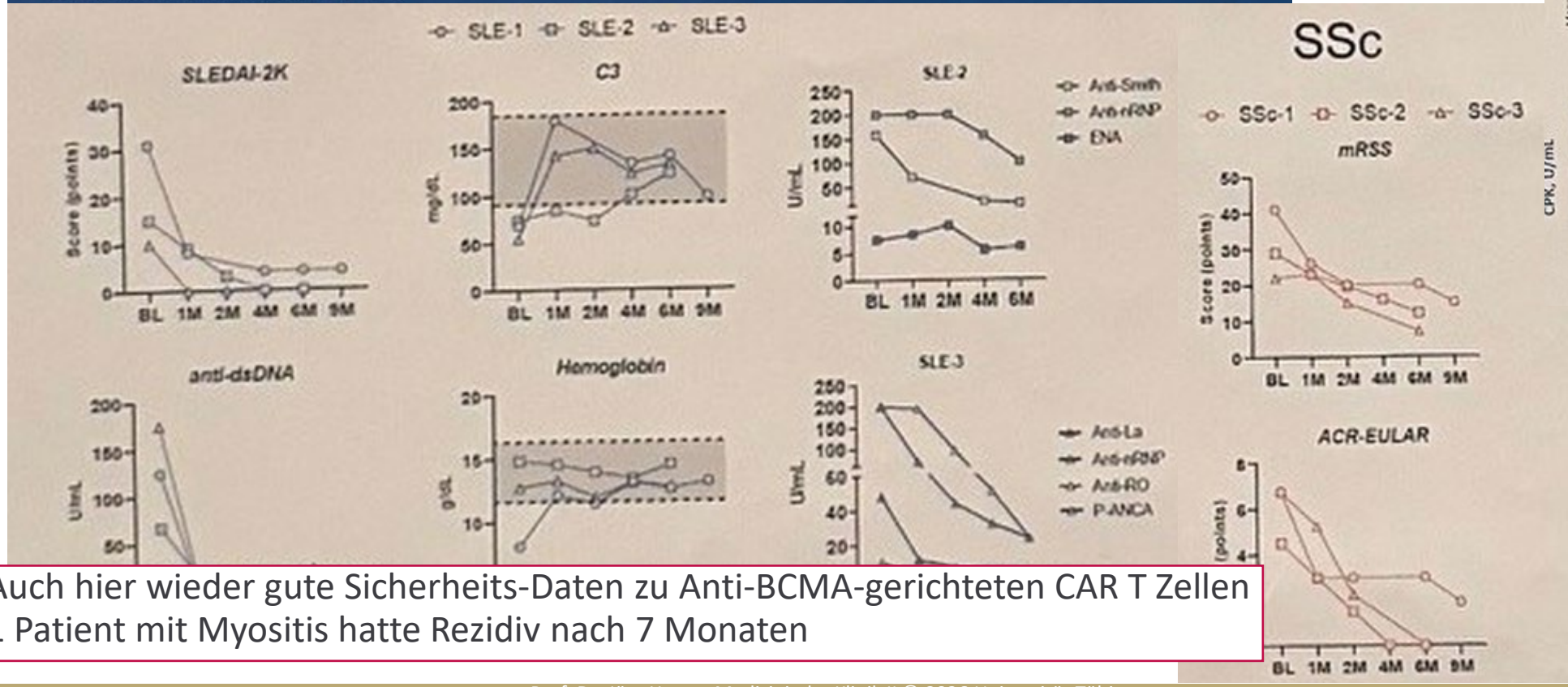

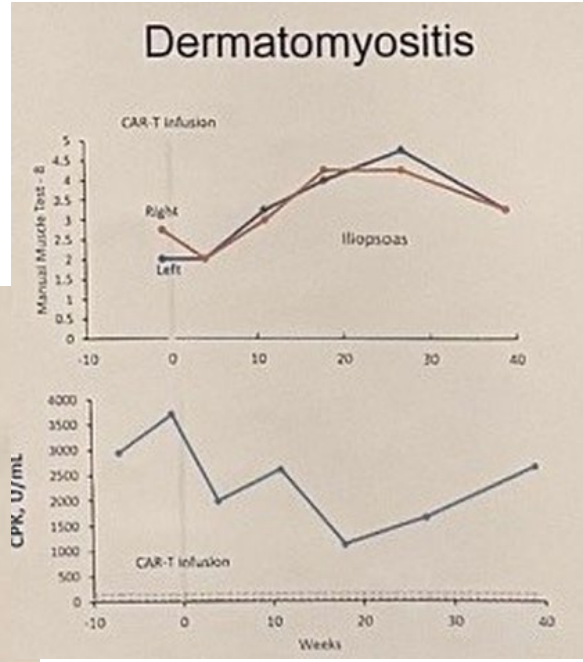

Auch hier wieder gute Sicherheits-Daten zu Anti-BCMA-gerichteten CAR T Zellen
1 Patient mit Myositis hatte Rezidiv nach 7 Monaten

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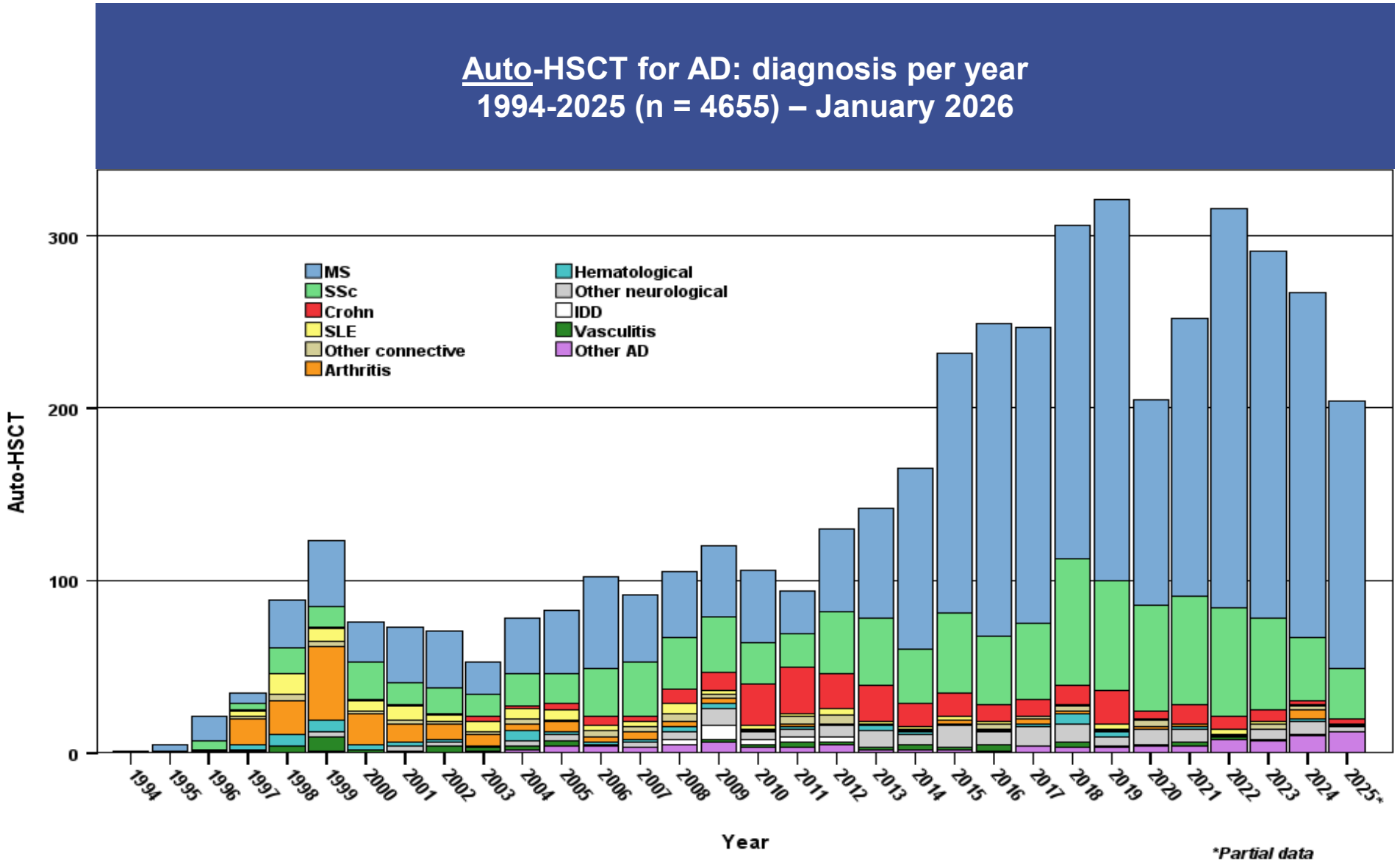
Autologe Stammzell-TX - Hintergrund



- aHSCT ist immer noch die meist verwendete Zell-Therapie bei Autoimmun-Erkrankungn
- Wir haben 30 Jahre Erfahrung!

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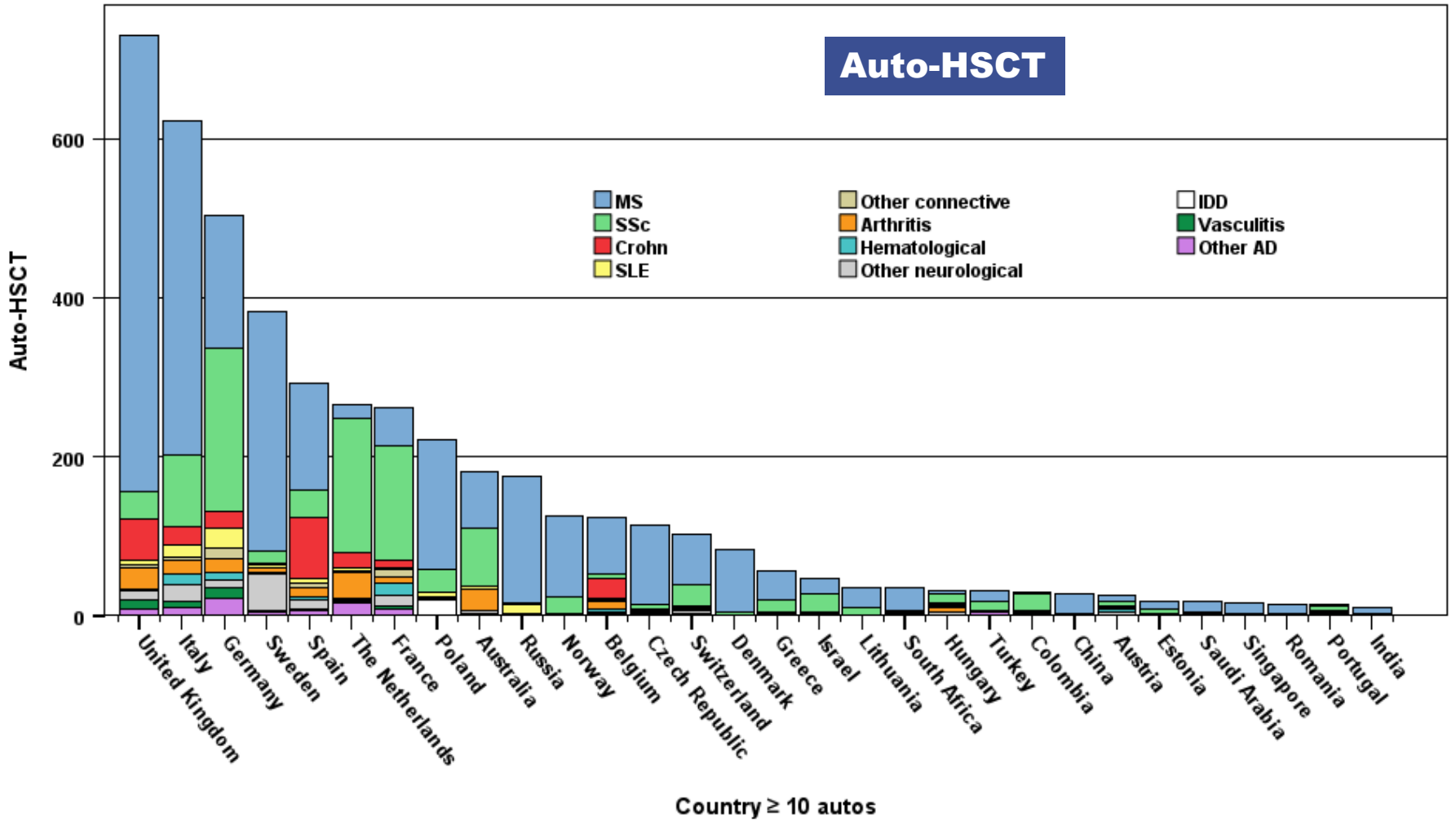
Auto-HSCT for AD / Country
1994-2025 (n = 4655) – January 2026



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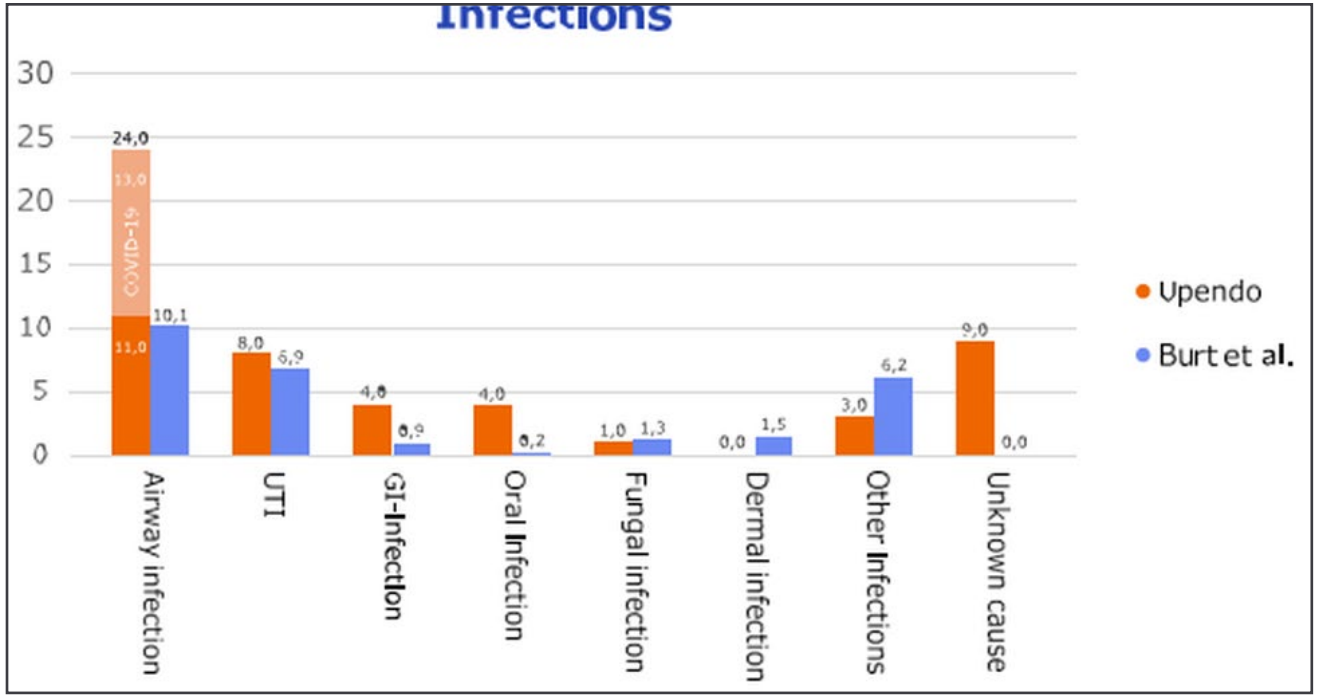
MS

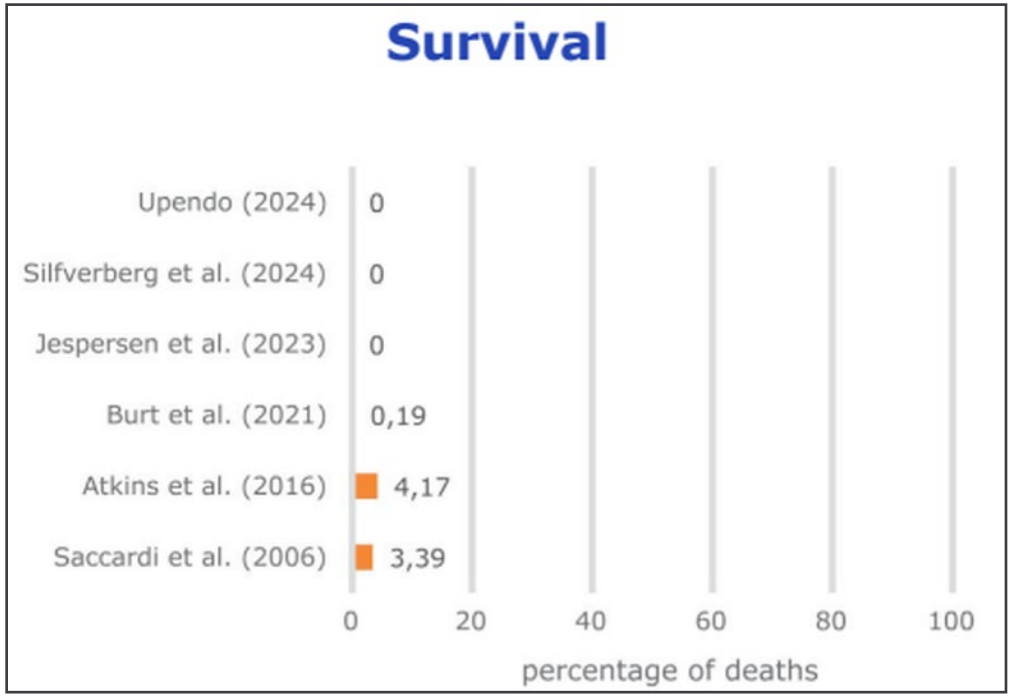
Infectious complications in dutch MS-patients within the first year after autologous hematopoietic stem cell transplantation in a foreign country

Lara Suzanne Oosterom[1], Sanne Haar[1], Aisa L Scheper[1], Melissa S Wolf[1], Goda CW Choi[2], Ellen PA Kramer[3,4], Gerald JD Hengstman[4]
1. Rijksuniversiteit Groningen, Groningen, Netherlands (the) 2. Univerity Medical Centre Groningen, Groningen, Netherlands (the)
3. Amsterdam University Medical Centre, Amsterdam, Netherlands (the) 4. Upendo, Boxtel, Netherlands (the)

- Hintergrund: Viele Patienten lassen sich aus Kostengründen oder Versicherungsgründen im Ausland transplantieren

Patient characteristics





Auch im Ausland Transplantierte profitieren von aHSCT bei MS, Infektionsraten und Mortalitätsraten sind gering

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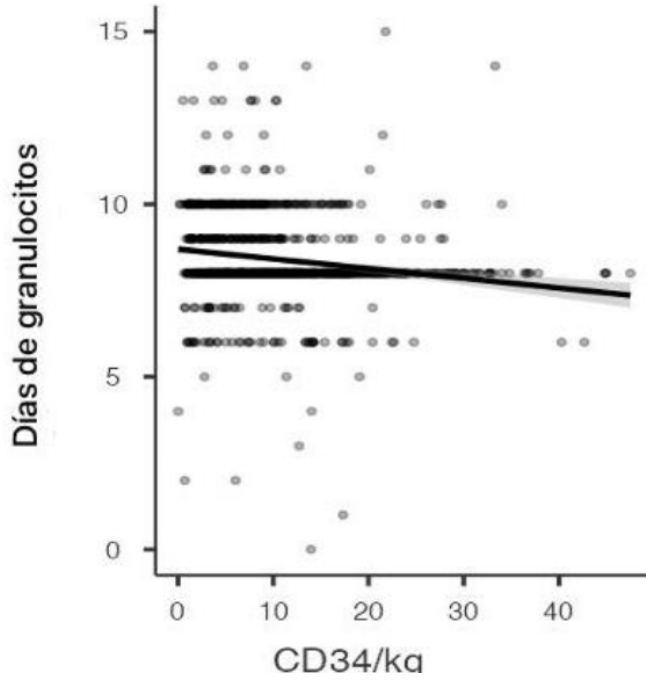
NUMBER OF CD34 CELLS EMPLOYED IN AUTOLOGOUS HEMATOPOIETIC STEM-CELL TRANSPLANTATION FOR PERSONS WITH MULTIPLE SCLEROSIS IS NOT RELATED TO THE NEUROLOGICAL OUTCOME OF THE PROCEDURE

Guillermo J. Ruíz-Argüelles[1], Michelle Lavoignet-Cisneros[1], Olivia Lira-Lara[1], Miguel Angel Viveros-Lugo[1], Sofía Chávez-Martínez[1], Monica Daniela Salgado-Cabrera[1], Lidia Joanna Alberto-López[1], Juan Carlos Olivares-Gazca[1], Max Robles-Nasta[1], Guillermo J. Ruiz Delgado[1].

[1] Centro de Hematología y Medicina Interna, Clínica Ruiz, Puebla, México



Retrospektive Analyse von 1417 MS Patienten welche in Mexiko transplantiert wurden.
Mobilisierung mit CYC + GCSF
Konditionierung mit CYC + Rituximab („Mexican method“)
Mediane CD34+ Zellzahl: 8.3 x 106 Zellen/kg

Ergebnisse:

- Mediane Zeit bis zur Granulozyten und Thrombozyten-Regeneration: 8 und 10 Tage
- Keine Korrelation zwischen der Anzahl der transplantierten Zellen und der Regeneration
- Kein Zusammenhang zwischen der Anzahl der transplantierten Zellen und dem klinischen Ansprechen

→ CYC/RTX scheint auch zu funktionieren bei MS
→ die Anzahl der transplantierten Zellen macht keinen Unterschied

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Clinical Characteristics, Prognostic Factors, and Transplant Outcomes in Hematological Malignancies with Behçet's Disease: A Retrospective Study of 114 Patient

Author: Xiaoyu Cao[1], Hui Fu[1,2], Mengzhu Zhao[3], Wenjun Wu[1,4], Ji-nuo Wang[1,4], Jian Yu[1,4]

1.Bone Marrow Transplantation Center of The First Affiliated Hospital & Liangzhu Laboratory, Zhejiang University School of Medicine, Hangzhou, China,

2.Department of Hematology, Taizhou Hospital of Zhejiang Province, Affiliated to Wenzhou Medical University, Linhai, China,

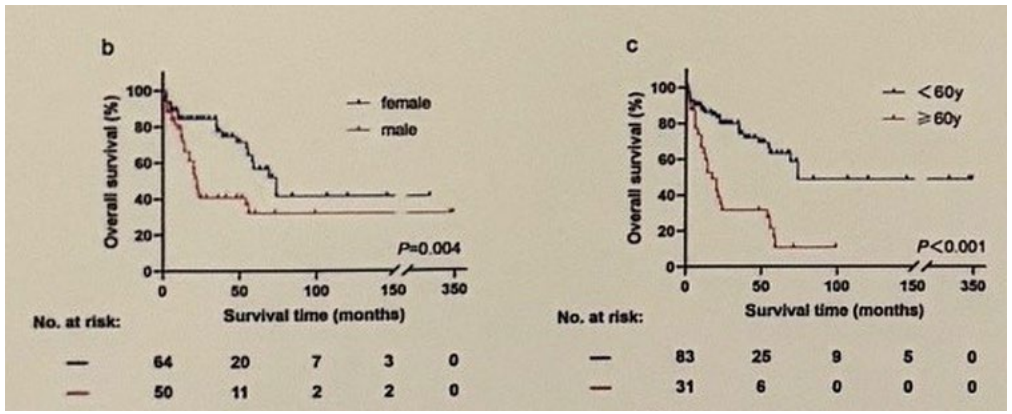
3.Department of Rheumatology, The First Affiliated Hospital, Zhejiang University School of Medicine, Hangzhou, China,

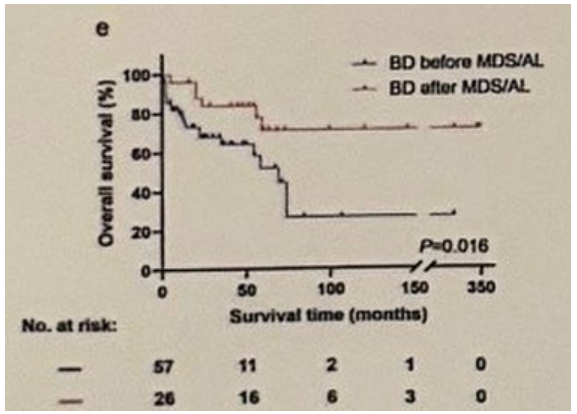
4.Institute of Hematology, Zhejiang University, Hangzhou, China

Retrospektive Analyse aller Patienten welche bei hämatologischer Grunderkrankung + Behcet stammzell-transplantiert wurden

88 x BD-MDS, 24 x BD-AML, 2 x BD-ALL

Männliches Geschlecht, Alter > 60 Jahre und BD Beginn vor der hämatologischen Erkrankung waren unabhängige Risikofaktoren für einen schlechteren Verlauf.





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Long-term evaluation of efficacy and safety after autologous stem cell transplantation for systemic sclerosis (NISSC1-LTE): a non-interventional cohort study from the Autoimmune disease Working Party (ADWP) of the European Society for Blood and Marrow Transplantation (EBMT)

J Henes, MC Oliveira, M Labopin, M Badoglio, HU Scherer, N Del Papa, T Daikeler, M Schmalzing, R Schroers, T Martin, G Pugnet, E W.A. Marijt, G Espinosa, M Rovira, R Greco, T Alexander, F Onida, Z Marjanovic, **D Farge**** For the Autoimmune Diseases Working Party of the European Group for Blood and Marrow Transplantation (EBMT)

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Background : NISSC1 Study

Autologous stem cell transplantation for progressive systemic sclerosis: a prospective non-interventional study from the EBMT ADWP

Table 1. Baseline characteristics of the patients (n=80).

Parameter	N (%) or median (range)
Patients	80
Age at aHSCT, years	43 (20-62)
Sex, female	57 (71.3)
Disease duration from SSc diagnosis, months	23.8 (5.3-103.7)

Table 2. Treatment regimen used for mobilization and conditioning.

Parameter	N (%) or median (range)
Mobilization	
CYC 2 g/m²	45 (57.7%)
CYC 4 g/m²	23 (29.5%)
Other dose	10 (12.8%)
Missing = 2	
G-CSF, yes	80 (100%)
Leukapheresis	
1	61 (77.2%)
2	11 (13.9%)
3	7 (8.9%)
CD34⁺ selection	
Yes	35 (43.8%)
None	45 (56.3%)
Conditioning regimen	
CYC 200 mg/kg	72 (90.0%)
CYC other dose mg/kg	4 (5.0%)
CYC 100 mg/kg + Thiotepa 10 mg/kg	4 (5.0%)
Rabbit ATG, yes	80 (100%)
Thymoglobulin (Sanofi/Genzyme), mg/kg	7.5 (2.5-7.5)
Grafalon (Neovi/Fresenius), mg/kg	40 (30-41)

Henes J ..., Farge D et al. Haematologica. 2021 Feb 1;106(2):375-383

Background : NISSC1 Study

Autologous stem cell transplantation for progressive systemic sclerosis: a prospective non-interventional study from the EBMT ADWP

After a 2 years follow-up:

100d and 2yrs TRM: 6.2 % (95% CI: 2.3-12.9)

Response to treatment: 83.8%

Event Free Survival (EFS):

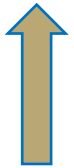
1. year: 83.6% +/-4

2. year: 68.7% +/-5

Real world data with very good results!
But what about long term response ?

- **mRSS**
  - **Baseline: 24 (2-49)**
  - **M12: 13 (0-40)**
  - **M24: 11 (0-32)**

- **FVC:**
  - **Baseline: 72.5 (43.0 – 132)**
  - **M12: 81.5% (46 -127)**
  - **M24: 82.0% (43 – 124)**



- **DLCO:**
  - **Baseline: 59.0% (18.9 – 120)**
  - **M12: 57.0% (31-123)**
  - **M24: 58.2% (22 – 117)**

Henes J …, Farge D et al. Haematologica. 2021 Feb 1;106(2):375-383

NISSc1 Long term extension: NISSc1-LTE

Aim:

To assess the long term safety and effectiveness of aHSCT for early severe or rapidly progressive Systemic Sclerosis (SSc) patients transplanted within the NISSc1 trial

Last patient included in NISSc1: February 2016

Total duration of follow-up: 10 years after autologous HSCT

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NISSc1-LTE

Primary end point: Progression free survival (PFS)

Survival since baseline without evidence of progression of SSc.

Progression: any of the following changes from baseline due to SSc:

- Death from SSc
- 10% drop in FVC and/or 15% drop in DLCO (of predicted values)
- 15% drop in LVEF by echo or cardio MRI
- 25% increase in modified Rodnan skin score (mRSS)

Secondary endpoints were:

- Overall survival (OS)
- Non-relapse mortality (NRM)
- Evolution of mRSS, FVC and DLCO values post aHSCT
- Late complications: malignancies, autoimmune diseases (AID), infections, major cardiac events (MACE)

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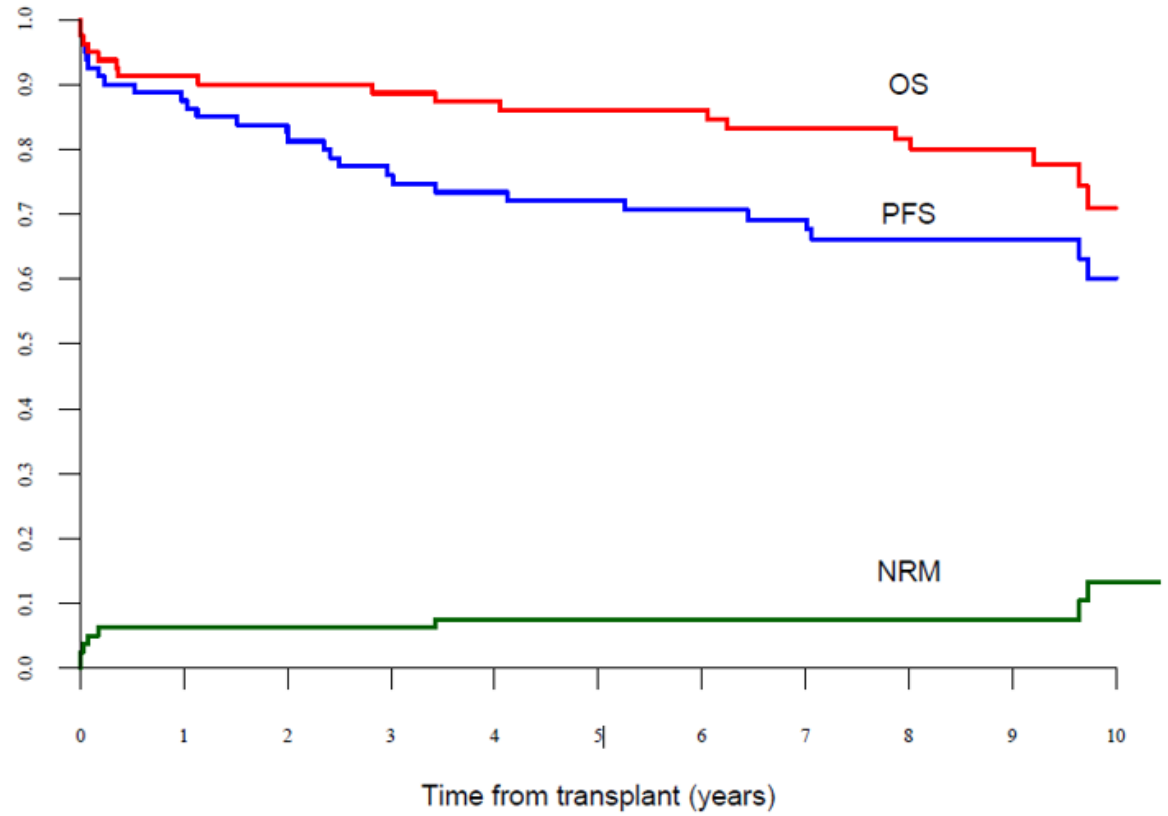
NISSc1 LTE - Results

Median follow-up was 9.2 (CI): 8.9-9.6) years after aHSCT



Patients at risk

OS	80	73	72	67	66	63	60	57	53	37	20
PFS	80	70	65	57	55	52	48	46	43	32	19
NRM	80	68	60	50	47	45	41	38	34	28	15

OS:

- 86% (95% CI: 76·2-92) at 5 yrs
- 71% (95% CI: 56·1-81·6) at 10 yrs

PFS:

- 72.1% (95% CI: 60·7-80·6) at 5 yrs
- 60.2% (95% CI: 46·5-71·4) at 10 yrs

NRM:

- 7.6% (95% CI: 3·1-14·8) at 5 yrs
- 13.3 % (95% CI: 5·6-24·4) at 10 yrs

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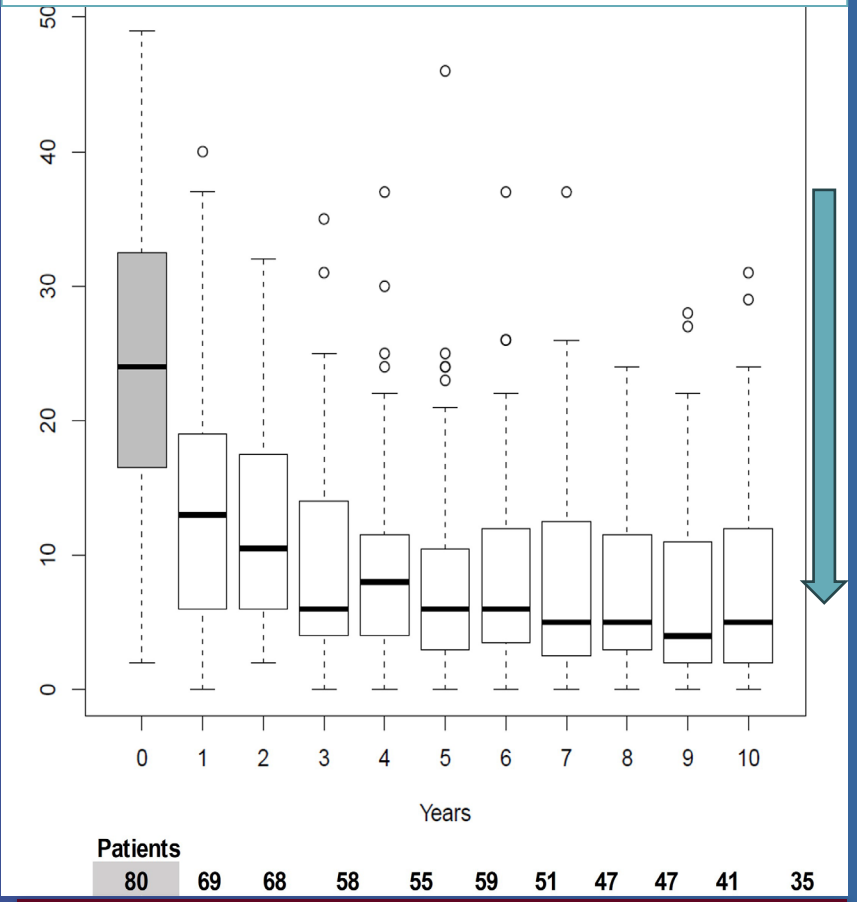
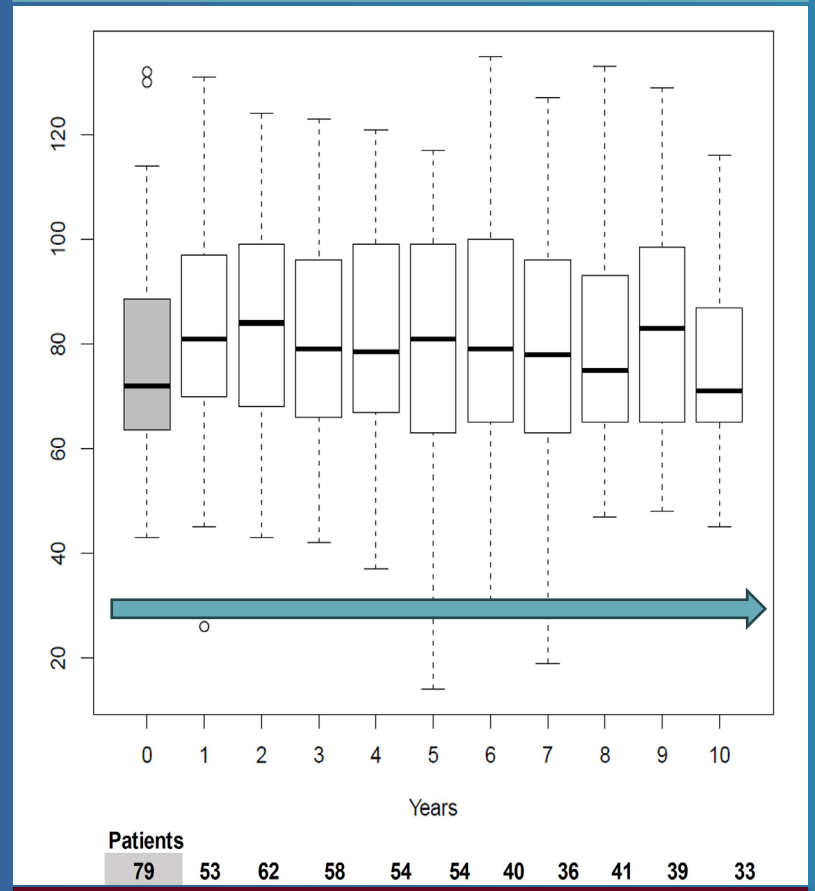
NISSc1 LTE – Results (median)

Skin: **modified Rodnan Skin score**



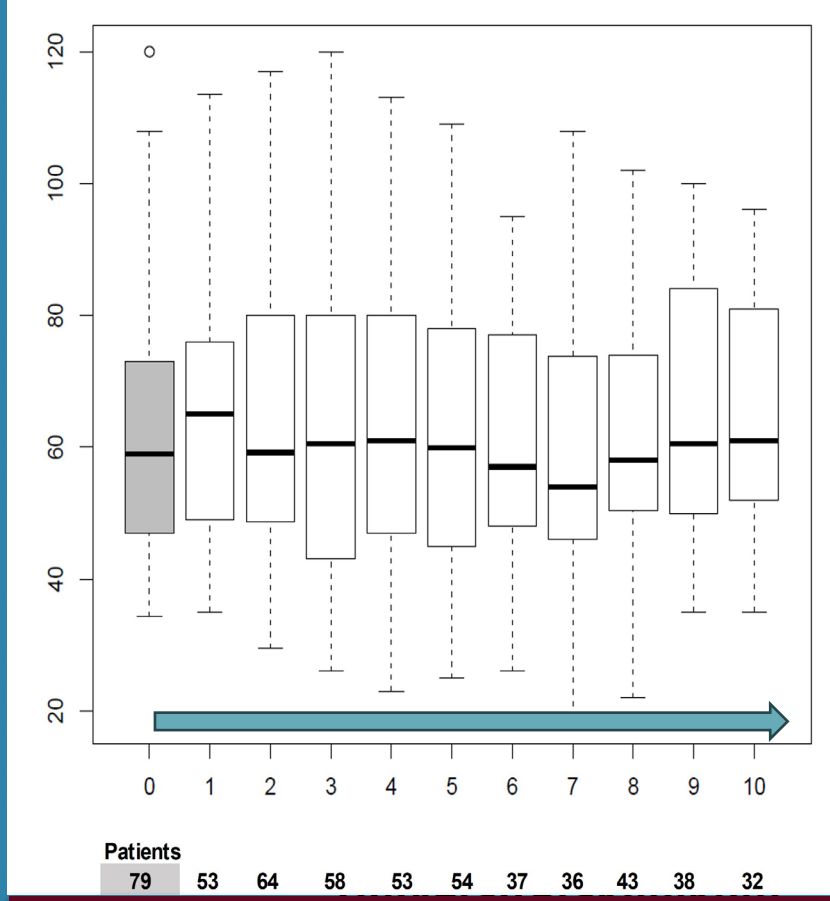
From 24 (2-49) at baseline
to 6 (0-46) at 5 yrs, and to 5 (0-31) at 10 yrs

Lung function: **FVC in % predicted**



From 72% at baseline
to 81% at 5 yrs, and to 71% at 10 yrs

Lung function: **DLCO in % predicted**



From 59% at baseline
to 61% at 5 yrs, and to 61% at 10 yrs

NISSc1-LTE Results: causes of death

5 transplant related , 9 progression, 2 post-aHSCT malignancy, 2 late infections

Long term post-transplant deaths	18 events (18 patients)	Time to event in months
- TRM, median (range)	5	0·33 (0·1-2·03)
- Early progression	3	4; 4; 13
- Late progression, median (range)	6	84·5 (33·7-110)
- Breast cancer	1	116
- Pancreatic cancer	1	48
- Endocarditis	1	115
- Gastrointestinal infection and sepsis	1	41

At 10 years, cumulative incidences of post-aHSCT malignancy was 8.3% at ten years

Long term post-transplant malignancies	6 events (5 patients)	Time to event in months
- Breast cancer	2	30; 90
- Lung cancer	1	71
- Pancreatic cancer	1	47
- Myelodysplastic syndrome	2	107; 121

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NISSc1 LTE: conclusion at 10 yrs

- **Longest follow-up period** of SSc patients after aHSCT in literature
- **Long-lasting effect** of aHSCT: **overall survival >70%** at 10 years
- **PFS of 60.2%** present: a novel contribution to literature
- **Disease-free status in** majority (>50%) for an entire decade
- 34/80 patients: **sustained remission without immunosuppression**
- I**ncidence of malignancy:** 8.3% → within expected range
- Results are an **important comparator** to all upcoming new (cellular) treatments

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Danke!

Mehr Rheuma?

POST EULAR: 01. Juli 2026

INDIRA Symposium: 28. November 2026