Identification and Description of Chronic Graft-Versus-Host Disease Patients, Using TriNetX LIVE™ Platform

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Background and Objective

Background

- Chronic graft versus host disease (cGVHD: one of the most common and severe complications of allogeneic hematopoietic stem cell transplant (allo-HSCT), affecting ~30 to 50% allo-HSCT recipients.
- Multi-organ autoimmune disease affecting patients' quality of life and contributes to non-relapse mortality,
- Yet **limited literature** describes **longitudinal disease characteristics and safety events in this population**.

Objective:

- **Identify cGVHD patients and describe their characteristics, organ involvement, and treatment patterns.**
- **Estimate safety events incidences, using TriNetX Global Network, a worldwide electronic health record research network.**

Methods

Cohort selection:

Patients aged ≥12 years with allo-HSCT and cGVHD diagnosis (ICD-10) between Jan 2021 and Dec 2025 were identified in TriNetX.

Descriptive analysis:

- Demographics at cGVHD onset (baseline), indication for allo-HSCT and treatments by line of therapy (LOT).
- Cumulative incidences of the safety events of interest, including liver disease, pneumonia, sepsis, cytopenia, and secondary malignancies.

Results

Baseline characteristics:

	Overall cGVHD (N = 6840)	cGVHD Ruxolitinib (N = 2500)	cGVHD Belumosudil (N = 880)
Demographics			
Age at index, mean ± SD	52.8 ± 17	53.1 ± 16.7	52.7 ± 16.6
Male at index, n (%)	3930 (57)	1450 (58)	510 (58)
Comorbidities, n (%)			
Heart disease	2880 (42)	1200 (48)	380 (43)
Liver disease	1320 (19)	640 (26)	180 (20)
Acute kidney failure, chronic kidney disease	2390 (35)	1060 (42)	350 (40)
Overweight and obesity	1160 (17)	490 (20)	150 (17)
Severe infections			
Streptococcal sepsis	100 (1)	40 (2)	20 (2)
Severe sepsis	420 (6)	240 (10)	140 (16)
Other sepsis	1060 (15)	530 (21)	70 (8)
Pneumonia	1270 (19)	610 (24)	210 (24)
Cytopenias, n (%)			
Anemia			
Anemia, unspecified	2650 (39)	1050 (42)	320 (36)
Anemia in chronic kidney disease	170 (2)	100 (4)	40 (5)
Neutropenia			
Neutropenia, unspecified	2530 (37)	890 (36)	190 (22)
Other drug-induced agranulocytosis	170 (2)	50 (2)	20 (2)
Thrombocytopenia			
Thrombocytopenia, unspecified	2920 (43)	1130 (45)	300 (34)
Other secondary thrombocytopenia	1010 (15)	430 (17)	110 (13)

- Blood cancer history before allo-HSCT (with potential multiple occurrences) included myeloid leukemia (55%), lymphoid leukemia (25%), myelodysplastic syndromes (23%), lymphomas (21%), and multiple myeloma (7%).

Affected organs at baseline / at last follow-up (up to 5 years) :

	Overall cGVHD (N = 6840)		cGVHD Ruxolitinib (N = 2500)		cGVHD Belumosudil (N = 880)	
Affected Organs, n (%)	**Baseline[a]**	**5-Year Follow-up[b]**	**Baseline[a]**	**5-Year Follow-up[b]**	**Baseline[a]**	**5-Year Follow-up[b]**
Skin and subcutaneous tissue	1160 (17)	3560 (52)	500 (20)	1340 (54)	160 (18)	510 (58)
Digestive system	2040 (30)	4480 (65)	790 (32)	1660 (66)	240 (27)	600 (68)
Mouth	490 (7)	1710 (25)	190 (8)	620 (25)	50 (6)	220 (25)
GI Tract						
Esophagus, stomach and duodenum	1000 (15)	2800 (41)	380 (15)	1050 (42)	130 (15)	410 (47)
Appendix	20 (0)	70 (1)	10 (0)	30 (1)	0 (0)	10 (1)
Noninfective enteritis and colitis	290 (4)	930 (14)	110 (4)	320 (13)	20 (2)	100 (11)
Other intestines diseases	770 (11)	2410 (35)	310 (12)	860 (34)	70 (8)	300 (34)
Liver	410 (6)	1400 (20)	180 (7)	550 (22)	50 (6)	190 (22)
Respiratory system	1690 (25)	4400 (64)	720 (29)	1620 (65)	270 (31)	620 (70)
Genitourinary system	1550 (23)	3960 (58)	660 (26)	1450 (58)	180 (20)	520 (59)
Musculoskeletal syst. connective tissue	1730 (25)	4620 (68)	760 (30)	1680 (67)	280 (32)	640 (73)

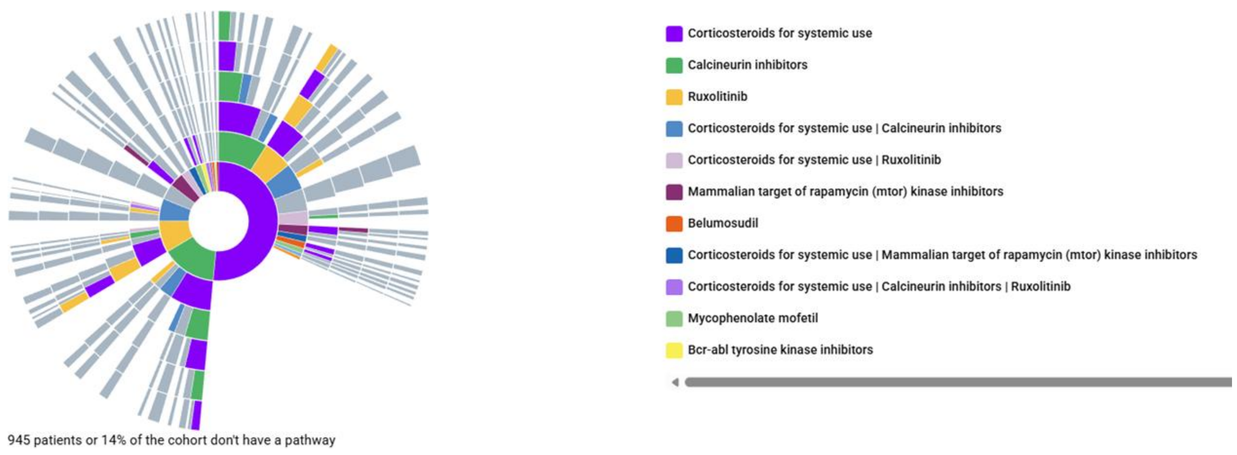
[a] Affected organs at baseline were assessed from the index date up to 30 days after.

[b] Affected organs at the 5-year follow-up were assessed from Day 31 after the index date, up to 5 years after.

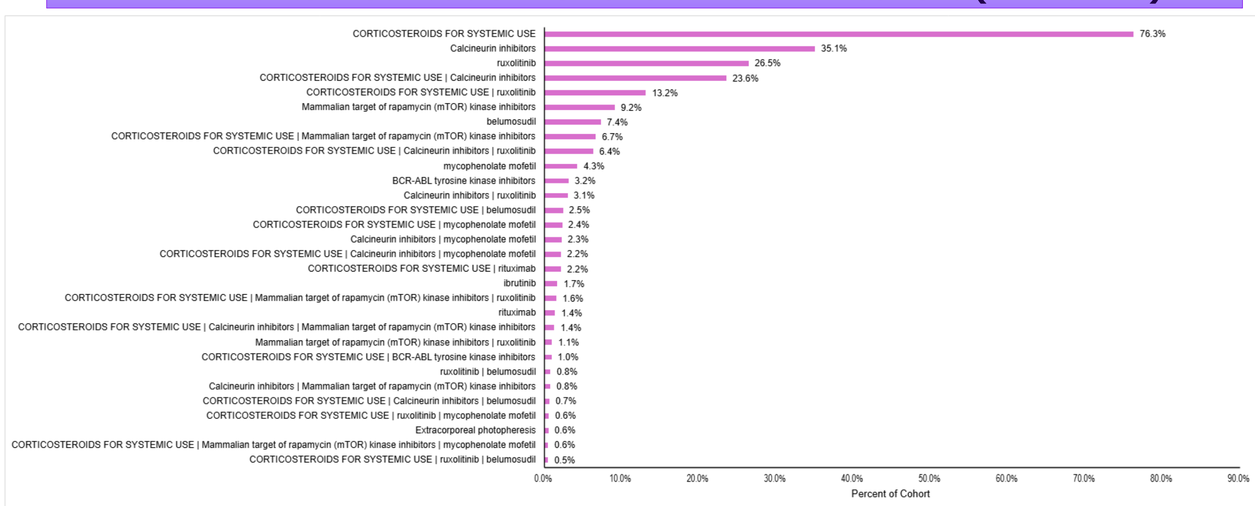
Treatment per LOT:

- Systemic corticosteroids dominated 1st LOT (52%) and dropped in subsequent LOTs (24 to 32% in LOT 2 to 6).
- Combination corticosteroids + CNIs was most used in 2nd LOT (12%), decreasing later (6-8%). Corticosteroids + ruxolitinib increased from early LOT to subsequent LOTs (2% to 4-6%).
- Ruxolitinib was prescribed in 49.24% of cases during the first four LoTs, with the remaining prescriptions occurring in later LOTs..
- Belumosudil was increasingly used from 3rd to 6th LOT (4% to 6%).
- Calcineurin inhibitors (CNIs) consistently used across all LOTs (13-17%).

Line of treatment sunburst for overall cGVHD cohort (N = 6840)



Treatments distribution for overall cGVHD cohort (N = 6840)



Safety events cumulative incidences

Anemia 63.3%; neutropenia 39.2%; thrombocytopenia 35.3%; severe infections, including pneumonia, 35.5%; pneumonia 29.8%; liver disease 25.1%; secondary malignancies 2.6%.

Discussion

- This feasibility study confirmed that TriNetX can reliably identify cGVHD patients and capture organ involvement, treatment pathways, and key safety events.
- The preliminary findings validated the data quality and supported moving forward with a full comparative study.
- The ongoing study will compare event rates across cGVHD patients treated with belumosudil and ruxolitinib using TriNetX Dataworks USA (2021–2025).

Conclusion

This study using TrinetX provides valuable insights on cGVHD patients and on safety in real-world settings. Observed patients' characteristics and treatment pathways align with a previous multicentric study in France (1). Observed LOTs also reflect adherence to the recent guidelines (2).

References

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