

Interactive Pocket Guide to NMOSD Therapeutics[†]

Healthcare Provider & Patient / Evolving Version 6.15.24 / © 2019-2024 Guthy-Jackson Charitable Foundation

FDA-Approved Therapeutics

Non-Approved (Off-Label) Therapeutics

Feature	Soliris [®]	Uplizna [®]	Enspryng [®]	Ultomiris [®]	Rituxan [®]	Actemra [®]	CellCept [®]	Imuran [®]
Medical Name	Eculizumab <i>(Alexion/AZ)</i>	Inebilizumab <i>(Amgen)</i>	Satralizumab <i>(Genentech)</i>	Ravulizumab <i>(Alexion/AZ)</i>	Rituximab <i>(Genentech)</i>	Tocilizumab <i>(Genentech)</i>	Mycophenolate <i>(Roche)</i>	Azathioprine <i>(Prometheus)</i>
Disease Target	Complement Protein C5	CD19+ B Cells	IL-6 Receptor	Complement Protein C5	CD20+ B Cells	IL-6 Receptor	T and B Cell IMPDH Enzyme	DNA & RNA Synthesis
Clinical Trial Name	PREVENT	NMOmentum	Sakura Sky Sakura Star	CHAMPION	RIN-1	TANGO	MONICA	TANGO
Trial / Study Publication	NEJM 2019	Lancet 2019	NEJM 2019 Lancet 2020	Ann Neurol 2023	Clinical Study Lancet 2020	Clinical Study Lancet 2020	Clinical Study Front Imm 2018	Clinical Study Lancet 2020
Clinical Trial Efficacy[†]	Yes; See Data (Mono/Poly)	Yes; See Data (Mono Tx)	Yes; See Data (Mono/Poly)	Yes; See Data (Mono/Poly)	Non-Trial Data (Mono/Poly)	Non-Trial Data (Mono/Poly)	Non-Trial Data (+ Steroid)	Non-Trial Data (Mono/Poly)
U.S. FDA Status	Approved Jun 27, 2019	Approved Jun 11, 2020	Approved Aug 17, 2020	Approved Mar 25, 2024	Not Approved [‡]	Not Approved	Not Approved	Not Approved
Package Insert	Soliris [®] FDA Label	Uplizna [®] FDA Label	Enspryng [®] FDA Label	Ultomiris [®] FDA Label	Rituxan [®] FDA Label	Actemra [®] FDA Label	CellCept [®] FDA Label	Imuran [®] FDA Label
Approved Indication	Anti-AQP4+ Age 18+ Yrs	Anti-AQP4+ Age 18+ Yrs	Anti-AQP4+ Age 18+ Yrs	Anti-AQP4+ Age 18+ Yrs	Not Approved	Not Approved	Not Approved	Not Approved
Medication Guide Summary	Soliris [®] Medication Guide	Uplizna [®] Medication Guide	Enspryng [®] Medication Guide	Ultomiris [®] Medication Guide	Rituxan [®] Medication Guide	Actemra [®] Medication Guide	CellCept [®] Medication Guide	Imuran [®] Medication Guide

Status in < 18 Yrs	Not Approved; Clinical Trial ; Used in HUS	Not Approved; Consult Peds Neurologist	Not Approved; Outcomes in Adolescents	Not Approved; Approved for HUS and PNH	Approved for NHL, RA, GPA ; MOGAD Data	Approved for SJI and PJI	> 3 months old; Consult Peds	Peds Data ; Consult Peds
Status in Pregnancy	Data Available ; Consult Neuro	Data Pending; Consult Neuro	Data Pending; Consult Neuro	Data Available ; Consult Neuro	Data Available ; Consult Neuro	Data Available ; Consult Neuro	Avoid Use (Fetal Harm)	Avoid Use (Fetal Harm)
Status in Comorbidity	Consult Neurologist	Consult Neurologist	Consult Neurologist	Consult Neurologist	Consult Neurologist	Efficacy Intact [see TANGO]	Consult Neurologist	Consult Neurologist

Safety Warning*	Box Warning: Nm Infection	No Specific Warning	No Specific Warning	Box Warning: Nm Infection	Box Warning: IRxn; TLS; PML	Box Warning: TB & Opp Infxn	Box Warning: Preg; Infxn; CA	Box Warning: Cancer; Infxn
Safety Precaution*	IRx; Infection; Nm Infection	IRx; Infection Ab; PMN	Liver Function; Cho; WBC; PLT	IRx; Infection; Nm Infection	IRx; Infection Ab; PMN	Liver Function; Cho; WBC; PLT	Infection; Tox; Malignancy	Infection; Tox; Malignancy
U.S. FDA REMS	REMS Required	REMS Not Required	REMS Not Required	REMS Required	REMS Not Required	REMS Not Required	REMS Not Required	REMS Not Required
Pre-Use Screening	None Required	HBV; JCV; TB Serum IgG	HBV; TB; Liver Function	None Required	HBV; JCV; TB Serum IgG	HBV; TB; Liver Function	Liver Function; Blood Cell Cnt	TPMT Enzyme Level
Required* Vaccines^Δ	FDA Required *see ACIP ^Δ	Standard *see ACIP	Standard *see ACIP	FDA Required *see ACIP ^Δ	Standard *see ACIP	Standard *see ACIP	Standard *see ACIP	Standard *see ACIP
Laboratory Monitoring	None Specifically Required	B Cell Count; Ig Levels; Neutrophils	Liver Function; Neutrophils; Lipid/LDL; PLT	PLT count; Liver / Kidney Function	Infection; B Cell Count; Total Ig; PMN	Liver Function; Chol & Lipids; WBC / PLT Cnt	Routinely Blood Cell Cnt; Bleeding Issues	Routinely WBC Count; Infxn; Cancer

Maintenance Dosing	Uniform Dose (1.2 g)	Uniform Dose (300 mg)	Uniform Dose (120 mg)	40/60/100 kg (2.4/2.7/3.0g)	Per Body m ² (~ 375mg/m²)	Uniform Dose (8mg/kg; 4wk)	Uniform Dosing (up to 2 g/d)	Per Body Mass (2-3 mg/kg/d)
Mode of Dosing	Intravenous Infusion	Intravenous Infusion	Subcutaneous Injection	Intravenous Infusion	Intravenous Infusion	Intravenous Infusion	Oral Tablet	Oral Tablet
Mono or Poly Tx	± Add On; Consult Neuro	± Add On; Consult Neuro	± Add On; Consult Neuro	± Add On; Consult Neuro	± Add On; Consult Neuro	± Add On; Consult Neuro	± Add On; Consult Neuro	± Add On; Consult Neuro
Induction Regimen	Every Week First 4 Doses	Every 2 Weeks First 2 Doses	Every 2 Weeks First 3 Doses	Loading Dose for 2 Weeks	Every 2 Week Half-Doses	Every 2 Weeks First 3 Doses	Loading Dose; Consult Neuro	Loading Dose; Consult Neuro
Routine Regimen	Every 2 Wks Infusion	Every 24 Wks Infusion	Every 4 wks Injection	Every 8 Wks Infusion	Every 24 Wks Infusion	Every Wk Injection	Usually Daily if Oral	Usually Daily if Oral
PLEX or IVIG Re-Dosing	Re-Dose < 60 min Post-PLEX	Consult Neurologist	Consult Neurologist	Re-Dose < 4 hours Post-PE/IVIG	Consult Neurologist	Consult Neurologist	Consult Neurologist	Consult Neurologist
Company Website	Soliris [®] Website	Uplizna [®] Website	Enspryng [®] Website	Ultomiris [®] Website	Rituxan [®] Website	Actemra [®] Website	CellCept [®] Website	Imuran [®] Website
Patient Support	OneSource [®] Website	Horizon BYS [®] Website	Access Solutions [®] Website	OneSource [®] Website	No NMOSD Aid Program	No NMOSD Aid Program	No NMOSD Aid Program	No NMOSD Aid Program

[†] Important: content is reference information only; it neither expresses nor implies recommendations for therapy. Consult the FDA-approved label.

* Label Box Warnings & Precautions per the U.S. FDA [2024]; consult the specific package insert for each particular therapy approved for use in NMO.

^Δ U.S. CDC Advisory Committee on Immunization in Persons with Altered Immune Competence (ACIP) [2021] Recommendations (www.cdc.gov/acip)

[‡] Approved for NMO by the Japanese Ministry of Health (Japan) but not by the FDA (U.S.)

Key: Tx = therapy; IRx = infusion reaction; REMS = Risk Evaluation & Mitigation Strategy; PLT = platelet; Nm = serious *N. meningitidis* infection; TB = tuberculosis; HBV = hepatitis B virus