

Atypical HUS: 2018 Therapeutic Drug Discovery

aHUS Alliance – Pharma Overview

Table 1

COMPANY	DRUG/Molecule	TARGET/Mechanism	FOCUS, Other
Alexion	ALXN 1210	longer-acting C5 inhibitor	aHUS PNH
	ALXN1210 SC	C5	Extended dose intervals
	ALXN1007	C5a	GvHD APS
	ALXN1102, ALXN1103 (TT30)	C3	PNH
	Soliris®/ eculizumab	C5	aHUS PNH More
Achillion	ACH-4471	Factor D	Focus: C3G, PNH, other info (XR)
	ACH-4471XR	Factor D	Extended Release, Tablet
	ACH-5228, ACH-5548	Factor D	Next-Gen Oral: Complement Diseases
ADIENNE Pharma & Biotech	MUBODINA®	C5	Focus: Typical HUS
	BEGEDINA®	CD26	GvHD
	Ergidina	C5	IRI

Akari Therapeutics	Coversin®	C5	PNH aHUS, GBS, MG Clinical Trial: PNH
	Coversin® Long Acting	C5 and LTB4	Other
	Coversin® Dual Acting	C5 and LTB4	Other
Alnylam	Cemdisiran(ALN-CC5)	C5	aHUS PNH
Amgen	ABP 959	C5 (Biosimilar to eculizumab)	aHUS PNH ANZCTR Trial
Amyndas Pharmaceuticals	AMY-101 C3	C3 (compstatin Cp40)	PNH C3G, Others
	AMY-201	C3	Other: mini-FH
	AMY-301	C3	AMD
Annexon	ANX005	C1q	Autoimmune IVIg Complement Mediated Disease

Apellis	Compstatin®/APL-2	C3	PNH: Paddock PNH: Pharaoh Glomerulopathies Other APL-2 Trials
Argenx - collaboration with Broteio	ARGX-113/ Efgartigimod ARGX-117	FcRn Novel target NHance™	MG, IgG-mediated autoimmune diseases complement-mediated indications
Attune	ATN-249 Unnamed	Kallikrein inhibitor oral Sm Molecules	HAE PNH complement mediated diseases
Bioverativ - a Sanofi company	BIVV009(formerly TNT009) BIVV020(formerly TNT020)	C1s mAb to activated C1s	CAD CAD
ChemoCentryx	Avacopan <i>(formerly CCX168)</i>	oral C5aR inhibitor	AAV C3G aHUS Other

Chugai - a ROCHE company	RG6107 - aka SKY59 - aka RO7112689	C5, SC	Complement mediated diseases
Genentech - a ROCHE company	SKY59 - aka RO7112689 - aka RG6107 Rituxan®/rituximab MPGN, IgAN, Other lampalizumab (RG7417)	C5, SC CD20 CT Terminated Jan 2018	Complement mediated diseases RA, NHL, CLL, GPA and MPA AMD, GA
Genentech - Tanox (a Genentech subsidiary)	TNX-558	C5a	Inflammatory Disease, others
Genmab - also see Novartis (listed below)	Ofatumumab	CD20	chronic lymphocytic leukemia
Genzyme - also see Sanofi (listed below)	Thymoglobulin®, new indication Genzyme/Sanofi Research Pipeline		Kidney transplant rejection Fabry, MS, Gaucher Type 3, others
GlaxoSmithKline (GSK)	Benlysta® (belimumab) 3196165		SLE RA

	2831781	GM-CSF	OA, Autoimmune Disease
	Daprodustat	PHI	Anemia with Chronic Renal Disease
Greenovation	Moss-FH	Factor H, C3	C3G, aHUS and PNH, aHUS Alliance Interview
InflaRx	IFX-1 / IFX-2	C5a	Complement inhibition: Sepsis Hidradenitis suppurativa AAV, autoimmune/ inflammatory
Inflazyme	Mirococept®/APT070	C3 convertase inhibitor	IRI, DGF
ISU Abxis	ISU305	Biosimilar, C5 inhibitor	PNH
LFB Group	hCFH	Factor H Anti-cd303	aHUS SLE, autoimmune diseases
Novo Nordisk / G2 Therapies	Neutrazumab	C5aR	SLE, RA, other
Novartis	LFG316	C5	PNH
<i>- also see Sandoz (listed below)</i>	KRP203	S1PR	Transplant Assoc Microangiopathy GvHD, SCLE

	CFZ533	CD40	Renal Transplant MG
Novartis	Ofatumumab	CD20	chronic lymphocytic leukemia
<i>- also see Genmab (listed above)</i>			
NovelMed	unnamed	C3b and C5b-9	PNH, aHUS, Others
	Bikacimab	Factor B	AMD
	NM9405	Properdin	PNH
Noxxon Pharma	NOX-D15	C5a	Complement Diseases
Omeros	OMS721 (IV and SC)	MASP-2, Lectin pathway	aHUS HCT-TMA IgAN Others
	OMS906	MASP-3, Alternative pathway	PNH, aHUS, AMD Others
Ophotech	Zimura (ARC1905)	C5	AMD, GA
Ra Pharma	RA101495	C5	PNH
	RA101495SC	C5	PNH, aHUS and LN

	RA101495 XR	C5	not specified
	Unnamed	Factor D, SC	C3GN and DDD, AMD
	Unnamed	C5, oral	PNH, gMG, and LN
	Unnamed	C1s	CAD, SLE, GBS, others
Regenesance/Complement Pharma	Regenemab	C6	PNH, Myasthenia Gravis, Others
Resverlogix	apabetalone / RVX-208	BET, Sm molecule	CVD, DM, CKD, Other
ROCHE	SKY59	C5, SC	Complement mediated diseases
<i>- also see Chugai</i>	RO7112689		
<i>- also see Genentech</i>	RG6107		
	Rituxan®/rituximab	CD20	RA, NHL, CLL, GPA and MPA MPGN, IgAN, Other
	lampalizumab (RG7417)	CT Terminated Jan 2018	AMD, GA
Sandoz	see Novartis/Sandoz above	biopharmaceuticals	Biosimilar Pipeline
<i>- a Novartis division</i>			
Sobi	SOBI005	C5	PNH, aHUS
(Swedish Orphan Biovitrum AB)	SOBI003	Enzyme Replacement Therapy	MPS IIIA (CNS)

Sanofi	Various affiliations	Sanofi and Alnylam RNAi	Sanofi R & D
		Sanofi Acquires Bioverativ	Sanofi/Bioverativ Pipeline
		Sanofi / Genzyme	Sanofi/Genzyme Pipeline
True North (Bioverativ/Sanofi)	TNT009	C1s	Complement Mediated Disorders, CAD
Various Pharma	Cinryze	C1-INH	Therapy Target: HAE
	Berinert		
	Ruconest		
	Others		



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Note: Created by the aHUS Alliance as a snapshot of aHUS drug discovery information available to the general public in early 2018, this chart contains basic information and therefore should not be considered a comprehensive review of companies, or investigational drugs. Inclusion in this table is not an indicator of endorsement for companies, research teams or products, so any mention or omissions should not be considered relevant for investment or other purposes.