

Mouse/ <i>mdx</i>		8 weeks	8 weeks	Increased	Increased weight in TA, Gas, Quad, EDL. Increased CSA in EDL		EDL: increased force	EDL: no effect							
			4 weeks		Increased fiber size in TA										
Monkey/ cynomolgus	Domagrozumab (PF-06252616)	3-5 years	8 weeks		Axial and appendicular muscle mass increased (per MRI)										
Mouse/ <i>Sod1</i> ^{G93A} (ALS)	RK35	4 weeks	84-90 days (end-stage ~135)		Increased weight in Gas, Diaphragm, Quad, TA		Increased grip strength							Gas: reduced atrophy, preserved fiber diameter. Diaphragm integrity preserved	[11]
Rat/ <i>Sod1</i> ^{G93A} (ALS)		6 weeks	95 days (end-stage ~128)		Increased weight in Gas, Diaphragm, Quad and TA		Increased grip strength								
Mouse/ <i>A17</i> (OPMD)	RK35	12 weeks	10 weeks	Increased	Increased weight and CSA in TA and EDL. No effect in soleus		TA; Increased force	TA: No difference						TA: Reduced collagen I, III, IV deposits. No effect on intranuclear inclusion bodies.	[12]
		42 weeks	10 weeks	No difference	No difference in CSA or muscle weight of TA									TA; No effect on intranuclear inclusion bodies. Reduced collagen IV deposition	[13]
Mouse/ CB17-SCID	REGN1033	9 weeks	4 weeks	Increased	Increased weight in Gas and TA. Increased fiber size of Gas	No difference in fiber-type composition									[14]
Mouse/ C57BL/6		N/A	4 weeks		Increased weight in Gas and TA										
		N/A	3 weeks				TA: increased force	TA: no effect							
		24 months	3 weeks				TA: increased force	TA: no effect							
Mouse/C57BL/6/ (Dexamethason. atrophy)		11 weeks	2 weeks				TA: increased force	TA: no effect							
Mouse/ BALB/cAnNTac (wild type)	YN41 (ground)	12 weeks	6 weeks	Increased	Increased weight in TB, TA, EDL, Gas, Quad, plantaris. "Lower limb hindlimb muscle" increased CSA		Increased grip strength								[15]
	YN41 (microgravity)			Increased	Increased weight in TB, Gas, Quad, TA, Plantaris, Sol. "Lower limb hindlimb muscle" increased CSA		Increased grip strength								
Mouse/ C57BL6	muSRK-015P	10 weeks	4 weeks		Increased weight of Gas, EDL. Total CSA increased in plantarflexor group	Increased IIB fiber size, no effect on overall composition	EDL; no effect								[16]
Mouse/ <i>Smn17</i> (SMA)	muSRK-015P (with SMN-C1 splice modulator)	24 days ('late' restoration cohort)	4 weeks	No difference	Increased muscle mass in TA, Gas. 'Plantarflexor muscle group' increased CSA	Trending increase in fiber size of IIB. No difference in type I,	Plantarflexor group: Increased	No effect on plantarflexor							[17]

					Fiber size increased in EDL.		force (twitch force)									
							EDL: No effect (tetanic force)									
		10 weeks	No effect													
Mouse/ C57BL/6	RAP-031	8 weeks	5 weeks	Increased			Gas: increased force (twitch force)	Gas: no effect								[26]
							Gas: No effect (tetanic force)									
Mouse/ C57BL/6	RAP-031	3 months	8 weeks	Increased	Increased volume of Gas.		Gas: no effect	Gas: decreased force								[27]
Mouse/ <i>Act1^{H40Y}</i>	RAP-031	14 days	3½ months	Increased	Increased weight of Triceps, Gas, Quad. Overall larger myofiber size	Quad: oxidative fiber diameter increased. Diaphragm: glycolytic myofibers hypertrophy	EDL: no effect	EDL: no effect	Soleus: no effect	Soleus: no effect			No decrease in nemaline rod structures			[28]
Mouse/ <i>Neb cKO</i>	RAP-031	14 days	7 weeks	No effect	No effect. No difference in CSA, fiber size or weight	Not quantified							No effect			[29]
Mouse/ <i>Mtm1δ4</i>	RAP-031	2 weeks	Until death (mean 54 days for treated animals)	Increased	Increased weight in Gas, Quad, triceps	Quad; Hypertrophy only in IIB fibers. No fiber-type switch							Gross evaluation of diaphragm: unaffected by genotype or treatment			[30]
Mouse/ C57BL/6			Corresponding to <i>Mtm1δ4</i> life-span	Increased	Increased weight in Quad, triceps, no difference in Gas. Increased fiber size of Quad	Quad; Increased size: I, IIA, IIB. No fiber-type switch										
Mouse/ <i>Mtm1^{R69C}</i>	RAP-031	2 weeks	5½ months		Increased weight Gas. No difference in Quad and triceps. Increased average fiber diameter in Gas	IIB fibers increased size. No fiber-type switch										[31]
Mouse/ <i>mdx</i>	RAP-031	8 weeks	16 weeks	Increased	Increased weight in Quad, Gas, TA, Plantaris, EDL. No difference in Soleus. Increased small fiber profiles in EDL.	No fiber-type conversion in either EDL or soleus	EDL: no effect	EDL: decreased force	Soleus: decreased force	Soleus: decreased force			No effect			[32]
Mouse/ C57BL/6				Increased	Increased weight in Quad, Gas, TA, Plantaris and EDL. No difference in soleus		EDL: increased force	EDL: no effect	Soleus: increased force	Soleus: no effect						
Mouse/ <i>mdx</i>	RAP-031	3 months	8 weeks	Increased	Increased volume of Gas	No change in MHC isoforms	Gas: increased force	Gas: no effect								[33]
Mouse/ C57BL/6	ACE-031	8 weeks	4 weeks	Increased	Increased weight in soleus, plantaris, Gas, EDL	Soleus: Type I and II-fiber size increased										[34]
Mouse/ C57BL/6	sActRIIB	3 months	8 weeks	Increased	Increased volume in Gas.											[35]
Mouse / C57BL/6	sActRIIB-Fc, i.m.				Increased weight in Gas, TA, pectoralis, Quad											[36]
Mouse/ C57BL/10	sActRIIB-Fc	6 weeks	2 weeks	Increased	Increased weight in Gas, Quad, TA											[37]
Mouse/ <i>mdx</i>		8 weeks	5 weeks	Increased	Increased weight in EDL, Gas, TA, fiber size increased in TA		TA: no effect	TA: decreased force			No effect		No effect in histopathology. eMHC: no effect			

Mouse/ <i>mdx</i>	sActRIIB-Fc	2 years	6 weeks	No difference	Increased weight in Quad, Tri, diaphragm. Increased myofiber size in Diaphragm and Triceps									Fibrosis, lipid infiltration and hydroxyproline content decreased in limb and diaphragm Triceps: eMHC increased	[38]
Mouse/ <i>R6/2</i>	sActRIIB-Fc	5 weeks	6 weeks	No difference	Increased weight in Quad, Gas, TA. Increased fiber size of TA and Quad		EDL, TA: increased force								[39]
Mouse/ C57BL/10 Hypoxia atrophy model	sActRIIB-Fc	10 weeks	2 weeks followed by 2 weeks of hypoxia	Increased	Increased weight in Soleus, EDL, Gas, TA and Quad. Increased whole-muscle and single fiber size in EDL		EDL, increased force				Improved resistance to eccentric lengthening				[40]
Mouse/ C57BL/6 (orchidectomized)	sActRIIB-Fc	5-6 months	30 days		Increased weight in Gas, TA and Quad. size increased in TA										[41]
Mouse/ <i>mdx</i>	sActRIIB-Fc	6-7 weeks	7 weeks	Increased	Increased weight in Gas, soleus, Quad, EDL and TA							No effect	No visible differences in pathology on H/E. SDH stains without effect of treatment	[42]	
Mouse/ <i>Dysf^{-/-}</i>	ActRIIB-Fc	6 weeks and 7 months	4 weeks									Increased	Decreased fibrosis	[43]	
Mouse/ <i>mdx</i>	sActRIIB-mFc	3 weeks	12 weeks	Increased	Increased weight in Gas, Quad, triceps and TA		EDL: increased force	EDL: no effect				No effect		[44]	
Mouse/ <i>Cav3^{p104L}</i>	sActRIIB-Fc	6 weeks	2 weeks		Increased myofiber area in triceps and Quad									[45]	
Mouse/ wild type	ACE-2494, s.c.	8 weeks	4 weeks		Increased weight in TA, Quad, Gas and EDL. No effect of weight in soleus	Sol fiber size increased								[46]	
Mouse/ <i>SmnΔ7</i>	sActRIIB-Fc	Postnatally	15 days		Increased muscle weight of Gas, Quad and triceps									[47]	
Anti-ActRIIB-antibody															
Mouse/ SCID	BYM338	12 weeks	4 weeks	Increased			Gas: increased force								[48]
Mouse/ SCID	BYM338	10 weeks	4 weeks 6 mg/kg	Increased	Increased weight in TA, EDL and Gas, no difference in soleus.										[19]
			4 weeks 20 mg/kg	Increased	Increased weight in TA, EDL, Gas and soleus										
Mouse/ C57BL/6 (glucocorticoid-induced atrophy)		4 months old	2 weeks concurrent with dexamethasone	Increased	Increased weight and CSA of TA		TA: increased force								
			2 weeks post 3 weeks dexamethasone treatment		Increased weight in Gas, plantaris and TA										
Mouse/SCID		Young	5 weeks	Increased	Increased weight in Gas										
Mouse/ <i>Mstn^{Ln/Ln}</i>	CDD866	19-24 weeks	4 weeks	Increased	Increased muscle weight (not specified)										
Follistatin administration or overexpression															
Mouse/ <i>F66;Dysf^{-/-}</i> (LGMD2B)	Follistatin over-expression crossed with <i>Dysf^{-/-}</i> mice		8 months		Muscle mass decreased with age			EDL: decreased force			Eccentric injury performed but not described	Increased, then decline with age	Exacerbation of dystrophic features. Increased Evans Blue Dye (EBD) uptake	[43]	
Mouse/ <i>F66;mdx</i>					Muscle mass maintained							Dystrophic features not exacerbated, mild improvement			

Mouse/ <i>SMAΔ7</i>	Follistatin overexpression				Increased weight of Quad, none in Gas or triceps									[47]
Mouse/ C57BL/6	AAV-delivered follistatin i.m.	4 weeks	Evaluation at 180 days	Increased	Increased weight in TA, Gas, Quad, Triceps									[49]
Mouse/ <i>mdx</i>		3 weeks	5 months	Increased	Increased weight in TA, Gas, Quad, Triceps		Increased grip strength				Decreased	Myofiber size increased. Satellite cell markers: no diff		
		210 days	560 days				Increased grip strength					Fewer necrotic fibers and mononuclear infiltrates		
Monkey/ Cynomolgus	AAV-delivered follistatin i.m	Not specified	Single administration of three injections in right Quad		Increased fiber size in Quad		Quad: Increased force						Myofiber hypertrophy	[50]
Mouse/ C57B110	ACE-083, i.m.	Not specified	4 weeks		Physiological CSA and weight of TA increased, fiber hypertrophy		TA: increased	TA: no effect						[51]
Mouse/ Trembler-J (Charcot-Marie-Tooth)		7 months	4 weeks	No effect	Physiological CSA and weight of TA increased, fiber hypertrophy		TA: increased	TA: no effect						
Mouse/ <i>mdx</i>		4 weeks	4 weeks	No effect	Physiological CSA and weight of TA increased, fiber hypertrophy		TA: increased	TA: no effect			Reduced (not statistically significant)			
Mouse/ C57BL/6	Follistatin fusion-protein (FST288-Fc) i.m. or s.c.	9 weeks	4 weeks		Increased weight in Gas vs uninjected muscle									[36]
Mouse/ C57BL/6	Engineered Follistatin (FS-EEE-mFc) i.v. or s.c.	9-10 weeks	4 weeks	Increased	Increased weight in Gas, Quad, triceps and TA									[44]
Mouse/ <i>mdx</i>		3 weeks	12 weeks	Increased	Increased weight in Gas, Quad, Triceps and TA		EDL: increased force	EDL: no effect			No effect	Decreased necrosis and fibrosis in Quad, no difference in diaphragm		
Mouse/ <i>hSMN2^{+/+}; hSMNΔ7^{+/+}; Snn^{-/-}</i>	Recombinant human follistatin		Untill postnatal day 16		Some effect in TA and Gas but none at treatment day 16. No effect on CSA									[52]
Mouse/ C57BL/6; <i>FS I-I</i>	Follistatin overexpression (transgenic mice)		5, 9, 13 weeks		Increased mass, fiber no. and fiber area of EDL, Sol, Quad, TA.		Increased grip strength							[53]
Mouse/ <i>mdx;FS I-I</i>			5, 9 weeks		Larger muscles		Increased grip strength					Reduced macrophage infiltration and fibrous changes of diaphragm		
Mouse/ <i>Sod1^{G93A}</i> (ALS)	AAV9-delivered follistatin i.m.	40 days	Approximately 80 days		Increased muscle mass in injected and non-injected muscles. Increased myofiber number and diameter		Grip strength increased							[54]
Liver-mediated overexpression of dominant-negative myostatin (dnMSTN), sActRIIB, myostatin propeptide and GASPI														
Mouse/ MF-1 (wild type)	AAV8-mediated overexpression (propeptide)	Injected at 6 weeks of age	4- and 10-weeks post injections	Increased	Increased weight of TA and Gas, increased CSA of EDL and Sol	Increased size of type I, IIA and IIB fibers	EDL: no effect	EDL: no effect	Sol: Increased force	Sol: No effect				[55]
Mouse/ <i>Sma^{C/C}</i>	AAV-mediated systemic expression (sActRIIB)	4 weeks	8 weeks	Increased	Increased weight in Quad, TA, EDL, Gas. No difference in weight of soleus	TA: Increased IIA size. EDL: Increased IIA and IIB size, increased total fiber number. Soleus: No difference	EDL, TA increased force	EDL, TA: decreased force	Soleus; increased force	Soleus: no effect				[56]
	AAV-mediated systemic expression (dnMSTN)			Increased	Increased weight in Quad, TA, EDL, Gas and soleus. Increased fiber size in EDL and TA but not in soleus	TA: Increased size of IIA and IIB fibers.	EDL: increased force	EDL: decreased force	Soleus: no effect	Soleus: no effect				

						fibers. EDL: increased proportion of glycolytic fibers RT-PCR: Soleus: MHC-IB percentage decreased, IIX+IIB increased. EDL: Increased proportion of IIB, decreased IIX									
Mouse/ <i>Beh^{CC}</i> (Compact)			2 months		Increased weight in EDL		EDL: no effect	EDL: decreased force							[70]
Mouse/ <i>Beh^{CC}</i> (Compact)			3-4 months,		Increased weight in TA, Gas, Quad										[75]
Mouse/ <i>Cmpt</i> (Compact)					TA: Fibertype switch to IIB EDL; IIB hypertrophy. Sol: I and IIA hypertrophy and fiber-type switch										[76]
Mouse/ <i>Mstn^{-/-};mdx</i>			3, 6, 9 months (exam.)		Mean fiber diameter increased In TA		Improved forearm grip-force					No effect	Diaphragm: Reduced fibrosis		[77]
Mouse/ <i>Mstn^{-/-};mdx</i>			18 months (exam.)		Increased weight in triceps, pectoralis, Quad, Gas.		Improved forearm grip-force						Diaphragm: "Abundant myofibers"		[78]
Mouse/ <i>Mstn^{-/-}; dy^W/dy^W</i>			3 weeks	No difference	Larger muscle mass and fiber numbers in rectus femoris	Reduced type I fibers in rectus femoris							No effect on necrosis and inflammation or number of infiltrating cells. Less fat tissue vs <i>dy^W/dy^W</i> mice.		[79]
Mouse/ <i>Mstn^{-/-}; scgd^{-/-}</i> (LGMD2F)			36 weeks (exam.)	Increased									Hydroxyproline decreased vs <i>scgd^{-/-}</i>		[5]
Mouse/ <i>Cav3^{PHL}; MSTN^{Pro}</i>			18 weeks	Increased	Increased weight in Triceps and Quad. Myofiber size increased vs caveolin-deficient mice		Improved forearm grip-force								[45]
Mouse/dominant -negative myostatin			16 weeks (exam.)		Increased muscle weight of Gas., triceps, Quad. Increased CSA of Quad.										[80]

Detailed results from previous studies as presented in Table 1. Changes are presented compared to controls of the particular study. The same reference may be found in different rows if various modalities of myostatin inhibition have been applied. References are listed according to reference list below. Abbreviations: AAV; adeno-associated virus, ActRIIB; activin receptor type IIB, CSA; cross-sectional area, CNF; centrally-nucleated fibers, EDL; m. extensor digitorum longus, eMHC; embryonic myosin heavy chain, Gas; m. gastrocnemius, GRMD; golden retriever muscular dystrophy i.m.; intra-muscular injection, i.p.; intraperitoneal injection, LGMD; limb-girdle muscular dystrophy, MHC; myosin heavy chain, OPMD; oculopharyngeal muscular dystrophy Quad; m. quadriceps, s.c.; sub-cutaneous injection, SDH; succinate dehydrogenase, SMA; spinal muscle atrophy, Sol; m. soleus, TA; m. tibialis anterior.

Supplementary table 2: Detailed overview of published and unpublished clinical trials with myostatin inhibitors as per PubMed – U.S. National Library of Medicine and clinicaltrialsregister.eu and clinicaltrials.gov (access date February 23rd 2021).

Treatment	Sponsor	Condition	Phase of trial	Age at treatment/ years	N	Length of treatment / regime	Primary outcome	Secondary outcome	Result	Status	Reference
Neutralizing monoclonal antibodies											
MYO-029 (Stamulumab)	Wyeth	Healthy subjects	I	N/A	72	MAD	Safety and tolerability, PK, PD	N/A	Well tolerated	Completed	NCT# 00563810
		BMD, FSHD, LGMD (2A, 2B, 2C, 2D, 2E, 2I)	I/II	>18	116	6 months	Safety and tolerability	Biological activity (manual muscle test, QMT, TFT, pulmonary function test, subject-reported outcome, MRI, change in muscle mass, LBM)	AE. No effect in MMT, QMT, subject-reported outcome, LBM	Completed	[81] EudraCT# 2004-000622-67 NCT# 00104078
PF-06252616 (Domagrozumab)	Pfizer	Healthy subjects	I	19-61	73	Single and repeated administrations (3 weeks)	Safety and tolerability	PK/PD, total LBM	Well tolerated. Minor AE: Headache, fatigue, upper RTI, muscle spasms. Single i.v.: increased whole-body lean mass by 5,38 %, Repeat i.v.: muscle vol increased 4,49 %	Completed	[82] NCT# 01616277
		DMD	II	6-16	121	96 weeks	Safety and tolerability, mean change 4 stair climb	TFT, pulmonary function tests, muscle volume, PK/PD	No significant between-group differences in any secondary clinical endpoints, withdrawn	Terminated	[83] NCT# 02310763 Extension: NCT# 02907619
		LGMD2I (FKRP)	I/II	>18	19	MAD, 32 weeks	Safety and tolerability	Muscle strength, TFTs, pulmonary function, LBM, PK, PD Exploratory outcome: muscle fat fraction	Preliminary results on clinicaltrials.gov per February 23 rd , 2021	Completed	NCT#02841267
LY2495655 (Landogrozumab)	Lilly	Healthy subjects	I	N/A	64	Single dose and 8 weeks multiple doses	“Clinically significant effect”	PK, PD, thigh muscle volume	Well tolerated. Thigh muscle volume increased	Completed	[84] NCT# 01341470
		Advanced cancer	I	71	29	Dose-escalation for 8 weeks	Safety and tolerability	PK	Well tolerated. Increased muscle volume at two of administered doses.	Completed	[84] NCT# 01524224
		Pancreatic Cancer /cachexia	II	45-86	125	8 weeks, opportunity of extended treatment cycles of 4 weeks	Overall survival	Progression-free survival, tumor response, duration of response, LBM, TFT, PRO, pain	Terminated due to lack of effect and imbalance in death rates between treatment arms.	Completed /Terminated	[85] NCT# 01505530
		Older, weak fallers	II	75-99	201	20 weeks	Change in appendicular LBM	TFTs, gait speed, QMT, body composition, rate of falls, myostatin serum concentration	Appendicular LBM, TFTs, gait speed improved	Completed	[86] NCT# 01604408
		Osteoarthritis undergoing total hip replacement	II	>50	400	12 weeks	Change in appendicular LBM	Secondary: QMT, PRO, whole-body-composition	Dose dependent increase in appendicular LBM and decreased fat, not enough to reach threshold of primary objective	Completed	[87] NCT# 01369511
REGN1033 (Trevogrumab) / SAR391786	Regeneron/ Sanofi	Healthy subjects	I	>60	76, 60	SAD	Assessment of safety, tolerability, administration	N/A	Results not reported (both studies)	Completed	NCT# 01507402, NCT# 01720576
		Healthy subjects	I	>60	125	N/A	Change in total lean mass	Safety and tolerability, appendicular lean mass	Results not reported	Completed	NCT# 01910220
		Healthy subjects	I	18-65	28	Single dose	PK in two different formulations of drug	Safety and tolerability	Results not reported	Completed	NCT# 02741739
		Sarcopenia	II	>70	253	3 months	Change in total LBM	AE, appendicular lean mass, gait speed, SPPB, DXA-evaluated body composition, 6MWT, QMT, TFT	Results not reported	Completed	NCT# 01963598

		sIBM	II	45-75	N/A	N/A	Change in total lean mass	AE, TFT, 6MWT, 10MWT, QMT	Withdrawn	Withdrawn	NCT# 03710941
REGN2477 (Garetosmab, Activin A-antibody) alone and in combination with REGN1033	Regeneron	Healthy subjects	I	35-70	82	Ascending dose	Safety and tolerability	Thigh muscle volume, DXA-evaluated body composition, PK	Results not reported	Completed	NCT# 02943239
SRK-015 (Apitegromab)	Scholar Rock	SMA 2, SMA 3	II	2-21	58	1 year	Change from Baseline in the Revised Hammersmith Scale or Hammersmith Functional Motor Scale Expanded (HFMSSE)	N/A	Results not reported	Active per January 31, 2021	NCT# 03921528
GYM329 /RG6237	Chugai Pharmaceutical / Roche	Healthy subjects (limb immobilization)	I	18-39	48	4 week parallel group design	Thigh muscle strength	Safety and tolerability, PK, PD	Results not reported	Recruiting per January 31, 2021	NCT# 04708847
Soluble ActRIIB											
ACE-031 (Ramatercept)	Acceleron	Healthy subjects	Ia	52-58	48	SAD	Safety and tolerability	PK/PD, body mass evaluation by DXA and MRI	Well tolerated	Completed	[88] NCT# 00755638
		Healthy subjects	Ib	45-75	70	MAD, 3 months	Safety and tolerability	PK/PD	Adverse effect (epistaxis) Increased LBM and thigh muscle volume	Completed	NCT# 00952887
		DMD	II	9-10	24, 11 in extension	12 weeks	Safety and tolerability	PK/PD (MRI evaluation, bone mineral density, TFT))	Body mass, Bone mineral density MD improved vs baseline (BL) No difference vs placebo AE (telangiectasias, epistaxis)	Terminated	[89] NCT# 01099761 Extension: NCT# 01239758
ACE-2494		Healthy subjects	I	45-75	48	SAD	Safety and tolerability	PK/PD, DXA-evaluated body composition, thigh muscle volume evaluated by MRI	Development of anti-drug antibodies	Terminated	[90] NCT# 03478319
Follistatin-Fc											
ACE-083	Acceleron	Healthy subjects	I	45-73	58	Single or double doses of intramuscular injections	Safety and tolerability	PK/PD, MRI/DXA evaluation, QMT	Well tolerated RF, TA increased volume Muscle strength no diff	Completed	[91] NCT# 02257489
		FSH	II	>18	58	6 months	Safety and tolerability	PK, PD, QMT, TFT, QOL	Did not meet secondary endpoints	Terminated	NCT# 02927080
		Charcot-Marie-Tooth	II	>18	42	3 months, 6 months vs placebo followed by 6 months open label	Safety and tolerability, change in muscle volume estimated by MRI	PK/PD, Muscular fat infiltration, QMT, TFT, QOL, CMT examination score)	Did not meet secondary endpoints	Terminated	NCT# 03124459
Antimyostatin adnectin											
BMS-986089	Bristol-Meyers-Squibb / Hoffmann-La Roche	Healthy subjects	I	21-55	140	SAD, MAD	Safety and tolerability	Pharmacokinetics	Results not reported	Completed	NCT# 02145234
RG6202 / BMS-986089 / RO-7239361		DMD	Ib/II	5-10	43	MAD, 24 weeks	Safety and tolerability	Thigh contractive tissue, CSA, PK	No AE. Increased LBM	Terminated	NCT# 02515669 [92]
RO-7239361		DMD	II/III	6-11	166	48 weeks	Changes in North Star Ambulatory Assessment score	TFT, QMT, 6MWT, walk, run and stride velocity	Primary endpoint not achieved	Discontinued	NCT# 03039686 [93]
Anti-ActRIIB antibody											
BYM-338 (Bimagrumab)	Novartis	sIBM	II	45-78	14	Single dose	Change in muscle volume	Body composition, LBM, QMT, TFT, 6MWT	Increased thigh muscle volume and LBM 8 weeks after treatment	Completed	[94] NCT# 01423110 Extension: NCT# 02250443 (terminated early)

									Increased 6MWT 16 weeks after treatment		
		sIBM	IIb/III	Mean 69	251	52 weeks	Change in 6MWT	LBM, QMT, sIBM functional assessment, rate of falls, SPPB	Increased LBM and improved sIBM function assessment. 6MWT not improved	Completed	[95,96] NCT# 01925209 EudraCT# 2013-000705-23 Extension: NCT# 02573467 EudraCT# 2015-001411-12)
		Sarcopenia	II	65-86	40	Single infusion	Thigh muscle volume, intramuscular and subcutaneous fat tissue	Total LBM, QMT, TFT	Thigh muscle vol increased Walking speed increased in subgroup with slow walking speed	Completed	[97] NCT# 01601600
		Sarcopenia	II	>70	217 (Extension: 160)	6 months	Change from baseline in SPPB	Safety, tolerability, 6MWT, gait speed, total LBM	Increased appendicular skeletal muscle index and LBM from baseline in 700 mg treatment cohort. No functional improvement in publication [98]	Completed	[98] NCT# 02333331 EudraCT# 2014-003482-25 Extension: NCT# 02468674 Extension: 2015-000471-27
		Patients undergoing surgical treatment of hip fracture	IIa / IIb	Not reported	245 planned	Single infusion, 24 weeks follow up	Change in total LBM	Gait speed, SPPB, safety and tolerability, rate of falls	Results not reported	Completed	NCT# 02152761 EudraCT# 2013-003439-31
		Casting-induced muscle atrophy (healthy)		24	16	Single administration	Thigh muscle volume, change in Intramuscular and subcutaneous adipose tissue	QMT, Safety and tolerability	Thigh muscle vol increased Strength no diff		[99] No clinical trial ID specified in article
		COPD	II	63.7	67	Two doses over 24 weeks period	Change in thigh muscle volume	6MWT, PK	Thigh muscle volume increased. No difference in 6MWT	Completed	[100] NCT# 01669174
		Cancer cachexia (lung or pancreas)	II	Average 62	57	Single dose, 8 weeks follow up	Change in thigh muscle volume	Body weight, PK/PD, bone mineral density, LBM, physical activity levels	P-value not calculated, appears like no effect of treatment	Completed	NCT# 01433263
		Type II diabetes	II	18-75	68	Monthly administrations, time frame not supplied	Change in body fat mass	HbA1c change, PK, body weight change, insulin resistance	Results not reported	Completed	NCT# 03005288
Follistatin gene therapy											
AAV1.CMV.FS344	Children's Hospital/ Milo Biotech	BMD	I/IIa (no placebo control)	24-27	6	Single treatment, 6 months of observation	Safety, change in 6MWT	QMT of quadriceps, muscle histology	6MWT increased in four of six subjects, improved histological findings	Completed	[101] NCT# 01519349
		sIBM	I/IIa	Average 65,9	14	Single treatment including exercise encouragement, followed for 1-2 years	6MWT	TFT, Biopsy, western blotting)	Improvement in 6MWT vs controls, improved histological finding	Completed	[102]
rAAV1.CMV.huFollistatin344	Jerry R. Mendell / Milo Therapeutics	DMD	I/II	N/A	3	Single dose, 24 months follow-up	AE	6MWT, size of muscle fibers	Results not reported	Completed	NCT# 02354781
Antimyoostatin peptibody											
AMG-745 / PINTA 745	Amgen	Prostate cancer in patients treated by androgen deprivation therapy	I	71-73	54	4 weeks	AE, PK, DXA, QMT, SPPB, TFT	N/A	LBM increased, decreased fat mass. No effect on physical function or lower extremity strength	Completed	[103]
		Age-associated muscle loss	II	N/A	N/A	N/A	Thigh CSA	QMT, TFT, 6MWT PK	Withdrawn	Withdrawn	NCT# 00975104
		End stage renal disease, kidney disease,	I/II	18-85	51	12 weeks	Safety and tolerability, LBM change	LBM, appendicular lean mass, mid upper arm muscle circumference, TFT, 6MWT	No results posted	Completed	NCT# 01958970

		protein energy easting									
Myostatin inhibition (information on myostatin inhibitory strategy not available)											
BLS-M22 (oral capsule)	BioLeaders Corporation	Healthy subjects	I	19-55	37	4-5 weeks	Safety and tolerability	PK, immunogenicity, changes in muscle mass	No results posted	Recruiting	NCT# 03789734

References are listed according to reference list below. Abbreviations: 10MWT; 10-minute walking test, 6MWT; 6-minute walking test, AE; Adverse events, BMD; Becker Muscular Dystrophy, COPD; chronic obstructive pulmonary disorder, CSA; Cross-sectional area, DMD; Duchenne Muscular Dystrophy, DXA; Dual-energy X-ray absorption, FSHD; Facio-scapulo-humoral dystrophy, LBM; Lean body mass, MRI; Magnetic resonance imaging, N/A; Not available, PRO; Patient reported outcome, PD; Pharmacodynamics, PK; Pharmacokinetics, QMT; Quantitative muscle testing, QOL; Quality of life, sIBM; spontaneous inclusion body myositis, SMA; spinal muscle atrophy, SPPB; Short Physical Performance Battery, TFT; Timed function test.

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