

NF1 Clinical Pipeline: Interventional Drug Therapies

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LG Glioma		Trial ID	TARGET	Agent	Phase 0	Phase 1	Phase 1/2	Phase 2	Phase 3
DK, NL, ES		NCT01158651	mTOR	Everolimus (RAD001)					
		NCT01089101	MEK	Selumetinib (AZD-6244)					
		2012-003005-10	Tyr-Kin (Bcr-Abl) +Tubulin binder	Nilotinib + Vinblastine					
Plexiform		Trial ID	TARGET	Agent	Phase 0	Phase 1	Phase 1/2	Phase 2	Phase 3
Registration Trial (EU Trial: ES) (EU Trial: IT) Adults Children, Young Adults		NCT03231306	MEK	Binimetinib					
		NCT02407405	MEK	Selumetinib (AZD-6244)					
		NCT02096471	MEK	PD-901					
		NCT02101736	Multi kinase (VEGFR, c-KIT, c-Met)	Cabozantinib					
		NCT02177825	Multi kinase (Bcr-Abl, C-kit, PDGFR)	Imatinib					
		2009-016922-15	Multi kinase	Imatinib					
		2012-000869-21	Multi kinase	Imatinib					
		NCT00846430	Antiprolif, Antiangiogenic	Peg-Interferon α-2b					
		NCT00396019	Antiprolif, Antiangiogenic	Peg-Interferon α-2b					
		NCT00634270	mTOR	Sirolimus					
		NCT02390752	Multi kinase (KIT, CSF-1R, Flt3)	PLX3397					
		NCT02285439	MEK	MEK162					
		NCT01362803	MEK	Selumetinib (AZD-6244)					
		NCT01140360	Multi kinase	Imatinib					
		NCT02124772	MEK	Trametinib (GSK-212)					
	NCT01275586	Tyr-Kinase (BCR-Abl)	Nilotinib						
MPNST		Trial ID	TARGET	Agent	Phase 0	Phase 1	Phase 1/2	Phase 2	Phase 3
		NCT02986919	BET (BRD and Extra-terminal)	CPI-0610					
		NCT02584647	mTOR + RT-Kinase	Sirolimus + PLX3397					
		NCT01661283	mTOR + VEGF	Everolimus + Avastin					
		NCT02691026, 2015-004747-39	PD-1	Pembrolizumab					
		NCT02008877	mTOR + HSP90	Sirolimus, Ganetespib					
		NCT02700230		Vaccine					
Others		Trial ID	TARGET	Agent	Phase 0	Phase 1	Phase 1/2	Phase 2	Phase 3
All tumors All tumors Bone Bone Bone Bone CNS Tumors CNS Tumors Cognition Cognition Dermal Dermal Dermal GIST GIST JMML		NCT01885195	MEK	MEK162					
		NCT02639546, 2014-004685-25	MEK	Cobimetinib					
		NCT02718131		BMP-2 (bone graft)					
		2007-003835-22		BMP-2 (bone graft)					
		NCT01968590		Cholecalciferol (Vit. D)					
		NCT01553149	TNF alpha	Lenalidomide					
		NCT02415153	TNF-alpha	Pomalidomide					
		NCT02256124, 2013-003405-26	Na channel and Glutamine release	Lamotrigine					
		2012-005742-38	HMG-CoA Reductase	Simvastatin					
		NCT02728388	Photosensitizer (PhotoDyn Therapy)	Levulan					
		NCT01682811	Photosensitizer (PhotoDyn Therapy)	Levulan					
		NCT02839720	MEK	Selumetinib (AZD-6244)					
		NCT02315625	Multi Kinase + mTOR	Sunitinib + Everolimus					
		NCT03109301	MEK	Selumetinib					
		NCT03190915	MEK	Trametinib					

NF2 Clinical Pipeline: Interventional Drug Therapies

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NF2	Trial ID	TARGET	Agent	Phase 0	Phase 1	Phase 1/2	Phase 2	Phase 3
Meningioma	NCT02831257	mTOR	AZD-2014					
Meningioma	NCT02523014	FAK and SMO	GSK2256098 and Vismodegib					
Other	2011-001789-16	Multi Kinase Inhibitor	Sorafenib					
Other	2010-023508-28	Tyr-Kinase (BCR-Abl)	Nilotinib					
VS	NCT01767792	VEGF	Bevacizumab (Avastin)					
VS	NCT01125046	VEGF	Bevacizumab (Avastin)					
VS	NCT02129647	Multi kinase (VEGFR)	Axitinib					
VS	NCT01490476	mTOR	Everolimus (RAD001)					
VS	NCT01345136	mTOR	Everolimus (RAD001)					
VS	NCT02379754	HSP70	Gentamycin					
VS	NCT02415257	HSP70	Gentamycin					
VS	NCT03079999	NSAID agent	Aspirin					
VS (Beijing only)	NCT02934256	EGFR-TKi	Icotinib					
VS and Meningioma	NCT02282917	HDAC	AR-42					
VS and Meningioma	NCT01880749	mTOR	Everolimus (RAD001)					
All NF2 Tumors	NCT03095248	MEK	Selumetinib					