Project title/type	PI/contact	Tool category	Tool Description/Features	Tool Status	Publications	Funded by NTAP
Ras Reference Reagents Initiative	Dominic Esposito	Reagent	Collection of entry clones (both stop and no-stop versions) of Ras pathway genes (>180 genes)	Available. To learn more, click here		No
Ras Reference Reagents Initiative	Dominic Esposito	Reagent	Reagents for Producing Fully-Processed KRAS 4B Protein	Available. To learn more, click here	Gillette WK, et al., Sci Rep. 2015, 5:15916. doi: 10.1038/srep15916	No
Ras Reference Reagents Initiative	Dominic Esposito	Reagent	Collection of wild-type HRAS, NRAS, KRAS4a and KRAS4b genes, and mutant KRAS4b (N=36), KRAS4a (N=6), HRAS (N=7) and NRAS (N=7) genes. Available as Gateway entry clones, all are fully sequenced and have the same context to enable optimal correlation of phenotype with genotype.	Available. To learn more, click here		No
Ras Reference Reagents Initiative	Dominic Esposito	Reagent	Two sets of RAS cell lines: "RASless" mouse embryonic fibroblasts (MEFs), and patient-derived cancer cell lines that express mutant KRAS genes.	Available. To learn more, click here		No
Role of Cumulative Genetic Defects in NF1 Tumorigenesis	Margaret (Peggy) Wallace	Reagent	Polyclonal rabbit anti-neurofibromin: PcNFn27, Monoclonal rabbit anti-neurofibromin: McNFn27a and McNFn27b. Used for investigating tumor progression pathways in NF1	Contact PI		No
Heat Shock Factor 1 (HSF1) as a Modifier of NF1-Associated Tumorigenesis and a Potential Therapeutic Target	Susan Lindquist	Reagent	pBabe vector encoding EGFP and pBabe vector encoding dominant-negative MEK1 mutant (Ser218Ala and Ser222Ala)	Contact PI	Dai C, et al. 2012. Loss of tumor suppressor NF1 activates HSF1 to promote carcinogenesis. J Clin Invest 122(10):3742-3754.	No

Project title/type	PI/contact	Tool category	Tool Description/Features	Tool Status	Publications	Funded by NTAP
A Robust Plexiform Neurofibroma Model for Preclinical Drug Screening	Lu Le	Animal Model	Mouse model of pNF where disease formation is addresed at the level of cell of origin (Nf1-/-;R26R-LacZ- Luciferase DNSC). Can be used for compounds screening studies.	Available. To learn more, click here	Chen Z, Liu C, Patel A, Lia C-P, Wang Y, Lu L. Cells of Origin in the Embryonic Nerve Roots for NF1-Associated Plexiform Neurofibroma, Cancer Cell 2014, November, 26(5), 596-599.	Yes
Development of a Preclinical NF1-MPNST Platform Suitable for Precision Oncology Drug Discovery and Evaluation	Angela Hirbe	Animal Model	PDX models of MPNST that capture genetic diversity at a broad level.	Not yet available - in development. To learn more, click here		Yes
Novel Therapeutics in Malignant Peripheral Nerve Sheath Tumor (MPNST)	Ping Chi	Animal Model	PDX models of MPNST that focus on specific signaling pathways associated with disease formation.	Not yet available - in development. To learn more, click here		Yes
Cutaneous Neurofibroma: Models, Biology and Translation	Piotr Topilko	Animal Model	Mouse model of cNF from which disease formation is addressed at the level of cell of origin (purported to be Prss56-positive BC cells). Data from identification and characterization of the cell type(s), among the BC derivatives in the skin.	Available. To learn more, click here	Radomska KJ, Coulpier F, Gresset A, Schmitt A, Debbiche A, Lemoine S, Wolkenstein P, Vallat JM, Charnay P, Topilko P. Cellular Origin, Tumor Progression, and Pathogenic Mechanisms of Cutaneous Neurofibromas Revealed by Mice with Nf1 Knockout in Boundary Cap Cells.Cancer Discovery. 2018 Oct 22.	Yes
Cutaneous Neurofibroma: Models, Biology and Translation	Lu Le	Animal Model	Mouse xenograft model of cNF involving murine derived SKPs with HoxB7 and cell of origin lineage marker.	Available. To learn more, click here	Chen Z, Mo J, Brosseau JP, Shipman T, Wang Y, Liao CP, Cooper JM, Allaway RJ, Gosline SJ, Guinney J, Carroll TJ, Le LQ. Spatiotemporal Loss of NF1 in Schwann Cell Lineage Leads to Different Types of Cutaneous Neurofibroma Susceptible to Modification by the Hippo Pathway. Cancer Discov. 2019 Jan;9(1):114-129	Yes
Genetically engineered mouse model for plexiform neurofibroma	Wade Clapp	Animal Model	Mouse model based on Krox20;Nf1flox/−; Used for drug screening, dosing schedules for selumetinib, and patterns of resistance.	Available. To learn more, click here	Yang FC, Ingram DA, Chen S, et al. Nf1-dependent tumors require a microenvironment containing Nf1+/- and c-kit-dependent bone marrow. Cell. 2008;135(3):437-448. PMCID: PMC2788814.	No

Genetically engineered mouse model for plexiform neurofibroma	Nancy Ratner	Animal Model	Mouse model based on DhhCre-Nf1fl/fl ; Used for drug screening and gene expression analysis studies	Available. To learn more, click here	Wu J, Williams JP, Rizvi TA, Kordich JJ, Witte D, Meijer D, Stemmer-Rachamimov AO, Cancelas JA, Ratner N. Plexiform and dermal neurofibromas and pigmentation are caused by Nf1 loss in desert hedgehog-expressing cells. Cancer Cell. 2008 Feb;13(2):105-16. doi: 10.1016/j.ccr.2007.12.027.	No
Genetically engineered mouse model for plexiform neurofibroma	Robert Kesterson	Animal Model	Mutation specific model (Nonsense mutation (c.2041C>T; p.Arg681*) and Missense mutation (c.2542G>C; p.Gly848Arg)) for testing of therapeutics.	Available. To learn more, click here	Kairong Li, Ashley N. Turner, Min Chen, Stephanie N. Brosius,Trenton R. Schoeb, Ludwine M. Messiaen, David M. Bedwell, Kurt R. Zinn, Corina Anastasaki, David H. Gutmann, Bruce R. Korf, and Robert A. Kesterson. Mice with missense and nonsense NF1 mutations display divergent phenotypes compared with human neurofibromatosis type I. Dis Model Mech. 2016 Jul 1; 9(7): 759–767	No
Genetically engineered mouse model for MPNST	Karen Cichowski, Tyler Jacks	Animal Model	Mouse model based on transgenic Nf1 Nf1+/-; p53+/- ; used for drug screening, safety, and biomarker studies	Available. To learn more, click here	Cichowski K, Shih TS, Schmitt E, Santiago S, Reilly K, McLaughlin ME, Bronson RT, Jacks T. Mouse models of tumor development in neurofibromatosis type 1. Science. 1999 Dec 10;286(5447):2172-6.	No
The Use of Nf1 and Nf2 Mutant Mouse Strains in the Investigation of Gene Function and Disease Development	Tyler Jacks	Animal Model	Model of glioblastoma: Nf1 +/-; Trp53 +/-	Available. To learn more, click here	Reilly KM et al. 2000. Nf1;Trp53 mutant mice develop glioblastoma with evidence of strain- specific effects. Nature Genetics 26(1):109-113.	
Genetically engineered mouse model for cerebellar defects	Yuan Zhu	Animal model	Mouse model based on Nestin-CreER+; Nf1fl/fl or flox/+	Available. To learn more, click here	Kim E, Wang Y, Kim SJ, Bornhorst M, Jecrois ES, Anthony TE, Wang C, Li YE, Guan JL, Murphy GG, Zhu Y. Transient inhibition of the ERK pathway prevents cerebellar developmental defects and improves long-term motor functions in murine models of neurofibromatosis type 1. Elife. 2014 Dec 23;3. doi: 10.7554/eLife.05151.	No
Molecular Regulation of Endothelial Cells by NF-1	Kevin Pumiglia	Animal Model	Inducible knockdown of Nf1 in the vascular endothelium: CAD5-CreERT2/Rosa26-LSL-td-Tomato/NF1flox/flox	Contact PI		No
Neurofibromin Function in Chondrocytes	Florent Elefteriou	Animal Model	Mouse model to assess Nf1 loss of function in chondrocytes	Contact PI	Wang W, et al. 2011. Mice lacking Nf1 in osteochondroprogenitor cells displays skeletal dysplasia similar to patients with neurofibromatosis type 1. Human Molecular Genetics 20(20):3910-3924.	No

Identification of the Cellular and Molecular Mechanisms Underlying the Osseous Manifestations of NF1 in Murine and Human Systems	Feng-Chun Yang	Animal Model	NF1 skeletal mouse model with reduced bone mineral density, reduced calcium ossification, reduced bone turnover, and increased ratio of spinal canal area to vertebral body area	Contact PI	Xiaohua W, et al. 2011. The haploinsufficient hematopoietic microenvironment is critical to the pathological fracture repair in murine models of neurofibromatosis Type 1. Public Library of Science 6(9):e24917.	No
Somatostatin and CD26: New Approach for the Treatment of NF1 Tumors	Slawomir Antoszczyk	Animal Model	MPNST sciatic nerve sheath tumor mouse model in immune competent mice	Contact PI	Antoxzczyk S, et al. 2014. Treatment of orthotopic malignant peripheral nerve sheath tumors with oncolytic herpes simplex virus. Neuro Oncol, first published online January 26, 2014	No
Preclinical Mouse Models of Neurofibromatosis	Kevin Shannon	Animal Model	Model of myeloproliferative disorder: Mx1-Cre; $Nf1^{flox/flox}$	Contact PI	Le DT et al. 2004. Somatic inactivation of NF1 in hematopoietic cells results in a progressive myeloproliferative disorder. Blood 103(11):4243-4250.	No
In Vivo Models of NF-1: The Nervous System and Tumorigenesis	Luis Parada	Animal Model	Conditional knockout of Nf1 in neurons: Synapsin 1-Cre; $Nf1^{flox/flox}$; Nf1+/-; p53+/- cis	Available. To learn more, click here	Zhu Y et al. 2001. Ablation of NF1 function in neurons induces abnormal brain development of cerebral cortex and reactive gliosis in the brain. Genes & Development 15(7):859-876. Vogel KS, et al. 1999. Mouse tumor model for neurofibromatosis type 1. Science 286(5447):2176-2179.	No
Porcine model for evaluating neurofibromatosis type 1	Jill Weimer	Animal Model	Yucatan miniswine with Exon 42 deletion (NF1+/ex42del), produced via recombinant adeno-assisted virus mediated gene targeting and somatic cell nuclear transfer, that recapitulates molecular and phenotypic hallmarks of the human disease. Can be used for studying biology of NF type 1 disease pathogenesis and testing of therapeutics.	Available. To learn more, click here	White, K. et al. A porcine model of neurofibromatosis type 1 that mimics the human disease. JCI Insight. 2018 Jun 21; 3(12): e120402.	No
Porcine model for evaluating neurofibromatosis type 1	David Largaspaeda	Animal Model	Ossabaw miniswine with R1947 mutation (NF1+/R1947*) model, produced via gene editing by TALEN, that recapitulates molecular and phenotypic hallmarks of the human disease, for studying biology of NF type 1 disease pathogenesis and testing of therapeutics.	Available. To learn more, click here	Isakson, S. et al. Genetically engineered minipigs model the major clinical features of human neurofibromatosis type 1 Commun Biol. 2018; 1: 158.	No

Zebrafish models for neurofibromatosis type 1	Thomas Look	Animal Model	Zebrafish model of NF1 that recapitulates molecular and phenotypic hallmarks of the human disease, for studying biology of NF type 1 disease pathogenesis and testing of therapeutics.	Available. To learn more, click here	Shin J, et al. Zebrafish neurofibromatosis type 1 genes have redundant functions in tumorigenesis and embryonic development. Dis Model Mech. 2012 Nov;5(6):881-94.	No
Group II Metabotropic Glutamate Receptors as Potential Pharmaceutical Targets for Neurofibroma Formation	Michael Stern	Animal Model	Drosophila models, entailing D42>CaMKIIT287D, D42>CaMKIIT287A, D42>ala, DFaKCG1,D42>DFaK+, DFaKCG1, D42>CaMKIIT287D, D42>PI3KDN, CaMKIIT287D, D42>PI3K-CAAX, ala, DFaKCG1,D42>PI3K- CAAX, DmGluRA112b;D42>CaMKIIT287D, DmGluRA112b; D42>+	Contact PI	Lin C et al. 2011. The metabotropic glutamate receptor activates the lipid kinase PI3K in Drosophila motor neurons through calcium/calmodulin-dependent protein kinase II and the nonreceptor tyrosine kinase DFaK. Genetics 188:601-613.	No
Control of Growth Within Drosophila Peripheral Nerves by Ras and Protein Kinase A	Michael Stern	Animal Model	Drosophila models, entailing D42>PI3K-CAAX, DmGluRA112b;D42>PI3K-CAAX, D42>PI3KDN, Foxo21/Foxo25, Foxo21/Foxo25;OK6>PI3KDN, D42>PI3K- CAAX;Foxo+, OK6>PI3KDN, D42>Foxo+, D42>PI3K- CAAX;S6KDN, D42>S6KACT, D42>S6KDN, gli>RasV12, PI3K2H1;gli>RasV12, gli>PI3KDN,RasV12, gli>PI3K-CAAX, PI3K2H1/PI3KA;gli>PI3K-CAAX, MZ709>PI3K-CAAX, gli-RafF179, gli>RalV20, Akt4226;gli>PI3K-CAAX, Akt4226/+;gli>PI3K-CAAX, gli>PI3K-CAAX;Foxo+(f19-5), gli>PI3K-CAAX, Foxo+(m3-1), gli>PI3K-CAAX, GFP	Contact PI	Howlett E, et al. 2008. A PI3-kinase-mediated negative feedback regulates neuronal excitability. Public Library of Science Genetics 4 e1000277. Lavery W, et al. 2007. Phosphatidyl inositol 3-kinase and Akt nonautonomously promote perineural glial growth in Drosophila peripheral nerves. Journal of Neuroscience27:279-288.	No

Project title/type	PI/contact	Tool category	Tool Description/Features	Tool Status	Publications	Funded by NTAP
Improving Cognitive and Behavioral Function in NF1 Genetically Engineered Mice	David Gutmann	Method	PET technique to detect and evaluating promising agents for attention deficit in Nf1 mutant mice	Available. To learn more, click here	Brown JA, et al. 2011. PET imaging for attention deficit preclinical drug testing in neurofibromatosis-1 mice. Exp Neurol 232(2):333-338	No
Evaluation of the Effectiveness of Calmodulin Inhibitors for the Treatment of Neurofibromatosis Type 1-Associated Malignant Peripheral Nerve Sheath Tumors	Steve Carroll	Method	Orthotopic xenografting method for luciferase-tagged MPNST cells	Available. To learn more, click here	Turk AN, et al. 2011. Orthotopic xenografting of human luciferase-tagged malignant peripheral nerve sheath tumor cells for in vivo testing of candidate therapeutic agents. Journal of Visualized Experiments 49 Byer SJ, et al. 2011.	No
Interchromosomal Associations that Alter NF1 Gene Expression Can Modify Clinical Manifestations of Neurofibromatosis 1	Andrew Hoffman	Method	Associated chromosome trap (ACT) assay	Available. To learn more, click here	Ling J and Hoffman AR. 2011. Associated chromosome trap for identifying long-range DNA interactions. J Vis Exp 23(50) 2621.	No

Project title/type	PI/contact	Tool	Tool	Tool Status	Publications	Funded by
Plexiform Neurofibroma Model Systems for Preclinical Drug Screening	Ray Mattingly	Non-animal model	3D co-culture model of pNF	Available. To learn more, click here	Kraniak JM, Chalasani A, Wallace MR, Mattingly RR. Development of 3D Culture Models of Plexiform Neurofibroma and Initial Application for Phenotypic Characterization and Drug	Yes
TRAPping the metabolic adaptations of plexiform neurofibroma	Giorgio Colombo	Non-animal model	Computational model of TRAP1 at the level of unique allosteric binding sites.	Not yet available - In development. To learn more, click here		Yes
A 3D Cutaneous Neurofibroma Model for Automated High-Throughput Drug Screenings	Alice Soragni	Non-animal model	3D organoid model of cNF using patient derived tissues	Not yet available - in development. To learn more, click here		Yes
Deconstruction and Reconstitution of NF1 Cutaneous Neurofibromas	Ray Mattingly	Non-animal model	Cell culture model of cNF using patient derived tissues	Not yet available - in development. To learn more, click here		Yes
Exploring Neurofibromin Function in a Yeast Model of NF1	Aaron Gitler	Non-animal model	S. cerevisiaeLira strains: 1. 1Δ mutant, 2. ira2Δ mutant	Contact PI		No

Project title/type	PI/contact	Tool category	Tool Description/Features	Tool Status	Publications	Funded by NTAP
Perpetuating NF1+/- and NF1-/- plexiform neurofibroma-derived tumor cells through the generation of induced pluripotent stem (iPS) cells	Edu Serra	Biospecimens	Authenticated and characterized iPS cells from pNFs, which are a replenishable source of cells for studying biology of pNF and models generation. Available to order from CRMB.	Available. To learn more, click here		Yes
Development of a PNF Cellular Assay for HTS	Margaret (Peggy) Wallace	Biospecimens	Semi-immortalized, characterized cells of pNF which are a replenishable source of cells for studying biology of pNF. Available for ordering from ATCC.	Available. To learn more, click here	Li H, Chang LJ, Neubauer D, Muir D, Wallace MR, Immortalization of human normal and NF1 neurofibroma schwann cells. Laboratory Investigation 2016 Oct; 96:1105–1115	Yes
A Nerve Sheath Tumor Bank from Patients with NF1	Christine Pratilas	Biospecimens	High quality clinically annotated biospecimen library including tissues, cells, genomic data, and PDX available to the NF1 global research community	Available. To learn more, click here		Yes
Genetic Studies of Neurofibromatosis	Margaret (Peggy) Wallace	Biospecimens	Semi-immortalized, characterized cells of cNF , whose use is aimed at elucidating the underlying biology of cNF	Not yet available - In development. To learn more, click here		Yes
Leveraging human induced pluripotent stem cells (iPSCs) to determine the impact of patient-derived NF1 gene mutations on peripheral sensory neuron-driven Schwann cell growth	David Gutmann	Biospecimens	Isogenic human induced pluripotent stem cells harboring NF1-patient germline NF1 gene mutations, useful for studying effects of sensory neuorons driven Schwann cell growth.	Not yet available - in development. To learn more, click here		Yes

Modeling cNF with human Schwann cells via tunable and reversible control of NF1 protein	Gabsang Lee	Biospecimens	Model of human SC development with the ability to tunably control NF expression, for studying the underlying biology of cNF.	Not yet available - in development. To learn more, click here		Yes
Induced Pluripotent Stem Cells as Potential Therapeutic Agents in NF1	Jonathan Chernoff	Biospecimens	NF1 gene repair vector designed to replace the disrupted Nf1 allele in a mouse model by restoring exon 31.	Contact PI		Yes
Receptor Tyrosine Kinases as Targets for Treatment of Peripheral Nerve Sheath Tumors in NF1 Patients	Victor Felix Mautner	Biospecimens	MPNST cell lines: 1) 1507 (loss of heterozygosity in p53; splicing mutation in intron 23-1 and a deletion in exon 10a of NF1). 2) 1844 (loss of heterozygosity in NF1 region)	Contact PI	Reuss DE, et al. 2013. Sensitivity of malignant peripheral nerve sheath tumor cells to TRAIL is augmented by loss of NF1 through modulation of MYC/MAD and is potentiated by curcumin through induction of ROS. PLoS One 8(2):e57152	Yes
Molecular Mechanisms of Schwann Cell Proliferation in NF1	George Devries	Biospecimens	Immortalized human NF1 MPNST cell line: T265-2C	Contact PI	Lee PR, et al. 2004. Transcriptional profiling in an MPNST-derived cell line and normal human Schwann cells. Neuron Glia Biology 1(2):135- 147.	Yes

Project title/type	PI/contact	Tool category	Tool Description/Features	Tool Status	Publications	Funded by NTAP
Development of Patient Reported Outcomes System for Patients with NF1-Associated Plexiform Neurofibromas using Mixed Method Approach	Jin Shei Lai	Clinical	PRO measure in pNF developed using a mixed-methods approach, whose methodology may be used for intervention studies.	Available. To learn more, click here	Lai, J.S., Jensen, S.E., Patel, Z., Listernick, R., Charrow, J. (2017). Using a qualitative approach to conceptualize concerns of patients with neurofibromatosis Type 1 associated plexiform neurofibromas (pNF) across the lifespan. American Journal of Medical Genetics Part A, 173(1), 79-87. Patel, Z.S., Jensen, S.E., Lai, J.S. (2016). Considerations for conducting qualitative research with pediatric patients for the purpose of PRO development. Quality of Life Research, 25, 2193-2199.	Yes
Development of a Child Neurofibromatosis Type 1 Health Related Quality of Life Measure	Nancy Swigonski	Clinical	PRO measure of health related QoL in children with pNF,that can be used as an end-point in clinical trials or intervention studies.	Available. To learn more, click here	Nutakki K, Varni JW, Steinbrenner S, Draucker CB, Swigonski NL. Development of the pediatric quality of life inventory neurofibromatosis type 1 module items for children, adolescents and young adults: qualitative methods. J Neurooncol. 2017 Mar;132(1):135-143.	Yes
Development of a needs-based quality of life Patient Reported Outcome measure specific to patients with NF1-associated pNFs	Stephen McKenna	Clinical	PRO measure of need-based QoL in pNF, that can be used as an end-point in clinical trials or intervention studies.	Not yet available – in development. To learn more, click here		Yes
The Development and Validation of PRO Measures to Assess Pain in Individuals with NF1 and pNF	Pam Wolters	Clinical	PRO measure of pain intensity and pain interference in pNF, that can be used as an end-point in clinical trials or intervention studies	Not yet available - in development. To learn more, click here		Yes
High-Resolution Ultrasonography and Optical Frequency Domain Imaging for Measurement and Characterization of Cutaneous Neurofibromas in Patients with NF1	Scott Plotkin	Clinical	Method being developed to assess the rate of growth/change of cNF on a much faster time scale versus other methods such as calipers, 3D photography.	Not yet available - in development.		Yes

microRNA Gene Regulatory Networks in Peripheral Nerve Sheath Tumors	Subbaya Subramanian	Databases and datasets	microRNA profile of neurofibromas, schwanomas, and peripheral nerve sheath	Contact PI	http://www.oncomir.umn.edu/SMED/stat_query.php	No
Genetic Characterization of MPNST by High- Density SNP Array	Arie Perry	Databases and datasets	SNP array from MPNSTs	Contact PI	Yu J, et al. 2011. Array-based comparative genomic hybridization identifies CDK4 and FOXM1 alterations as independent predictors of survival in malignant peripheral nerve sheath tumor. Clinical Cancer Research 17(7):1924-1934.	No
Studies of Neurofibromatosis 1 modifier genes	Andre Bernaras	Databases and datasets	Beagle Database: a genome-wide multi-species biological annotation tool (incorporates published Ras superfamily database)	Contact PI	Bernaras, A. 2006. Ras Superfamily and interacting proteins database. Methods in Enzymology 407:1-9.	No
Biological Basis of Neurodevelopmental Deficits in NF: Insights Through Expression Profiling	Eric Hoffman	Databases and datasets	Microarray data set from the hippocampus of mice with Nf1 haploinsufficiency	Contact PI	http://pepr.cnmcresearch.org/browse.do?action=list_prj_exp&projectId=31	No