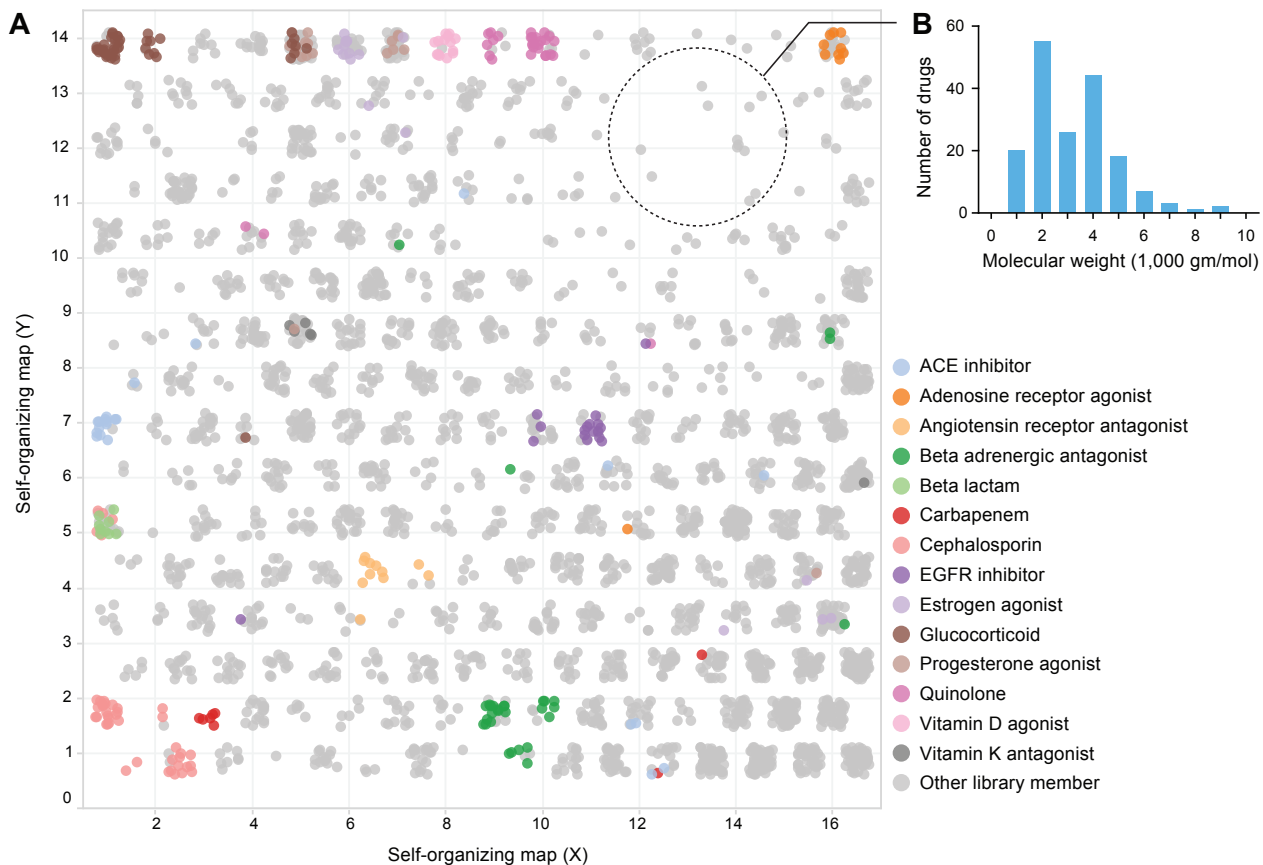


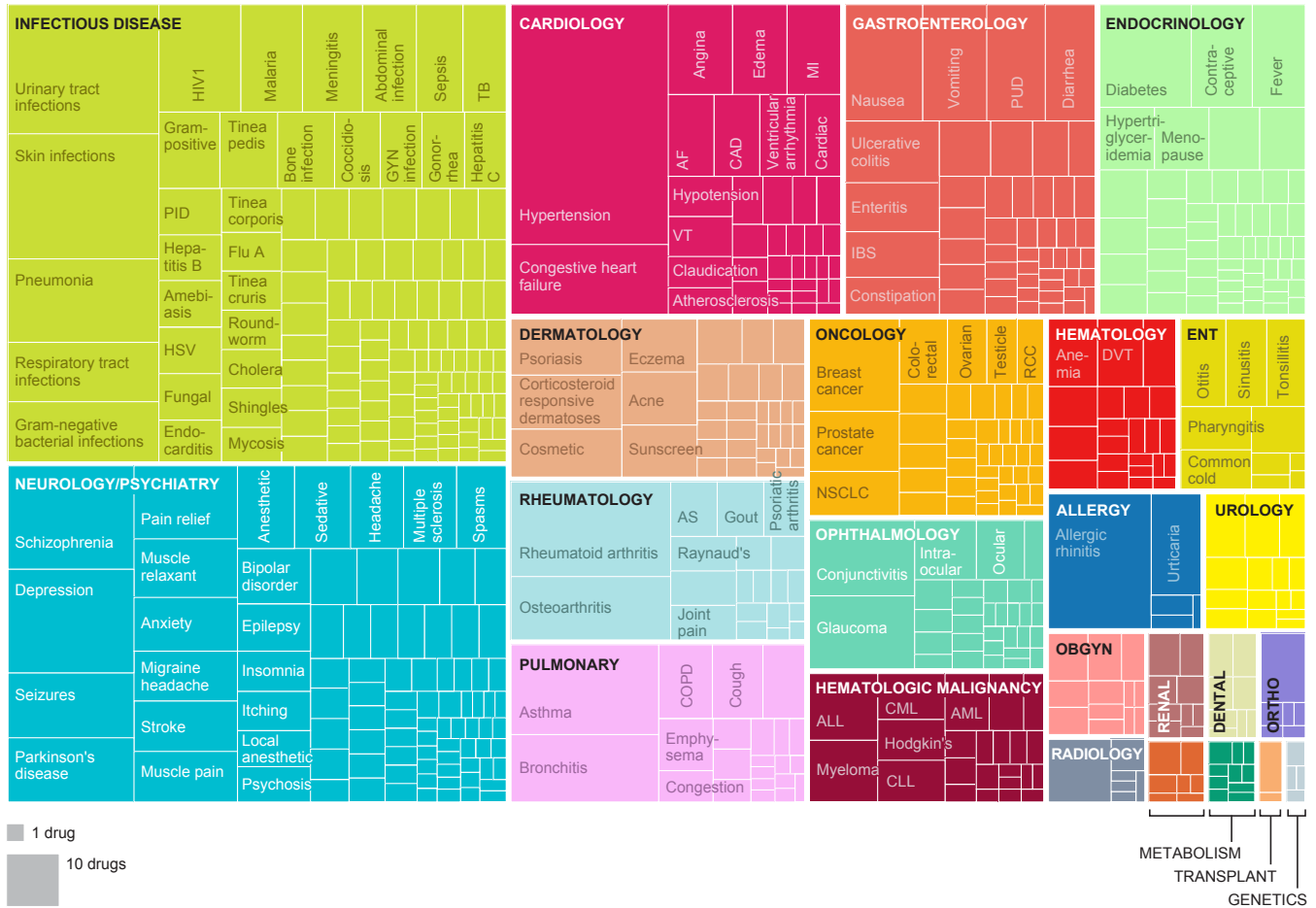
Figure S1



Supplementary Figure 1: Repurposing Library coverage of chemical structure diversity.

A) All identified clinical drugs are grouped into clusters using a self-organizing map of chemical fingerprints. Library members with known related features are indicated by color. B) Areas of reduced library coverage tend to include high molecular weight drugs. The molecular weight distribution for missing compounds within the designated map region is shown.

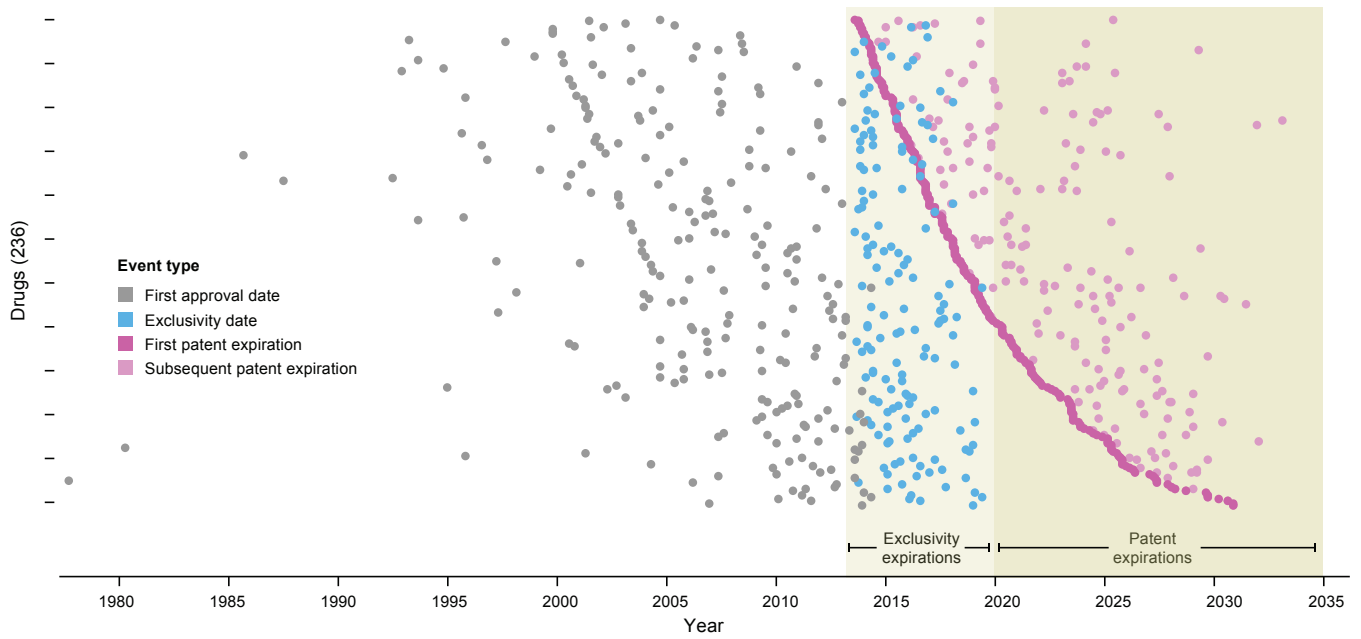
Figure S2



Supplementary Figure 2: Curated indications for launched drugs.

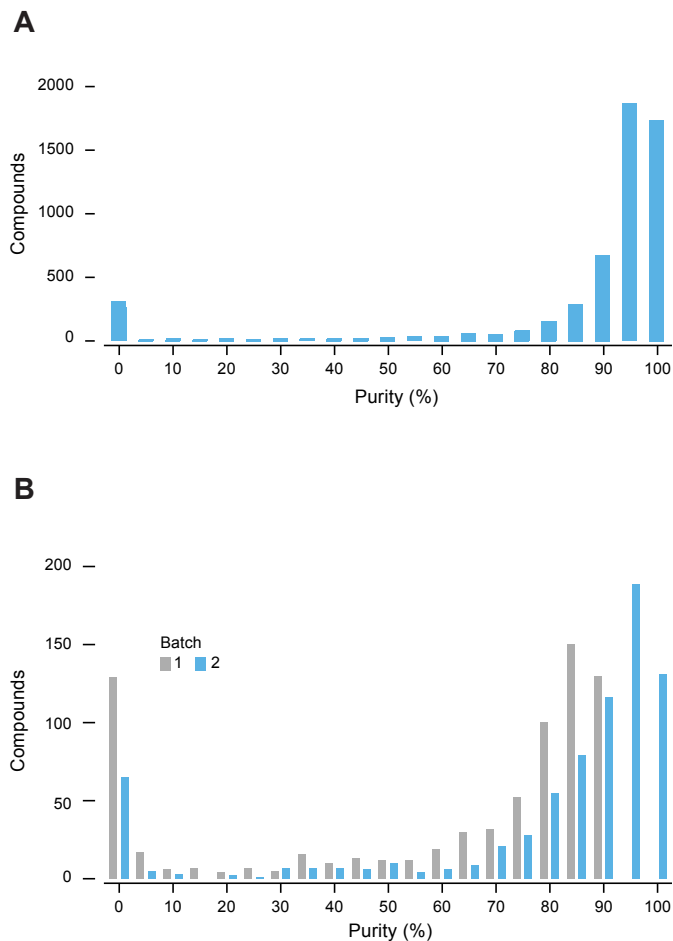
A total of 3,519 drug indication-pairs covering 1,918 drugs were obtained from official prescribing labels or other sources. Indications were mapped to a manually-curated vocabulary of 644 unique terms. The area shown for each indication is proportional to the number of corresponding drugs. Drugs with multiple approved indications appear more than once. Indication terms are grouped into one of 24 primary disease areas.

Figure S3



Supplementary Figure 3: Repurposing Hub coverage of drug patent and exclusivity information. Graphical overview of approval, exclusivity expiration, and patent expiration dates is shown. Drug ingredients were filtered to include only single-agent therapeutics and drugs covered by current substance patents. Rows are sorted by the first patent expiration.

Figure S4



Supplementary Figure 4: Library quality control.

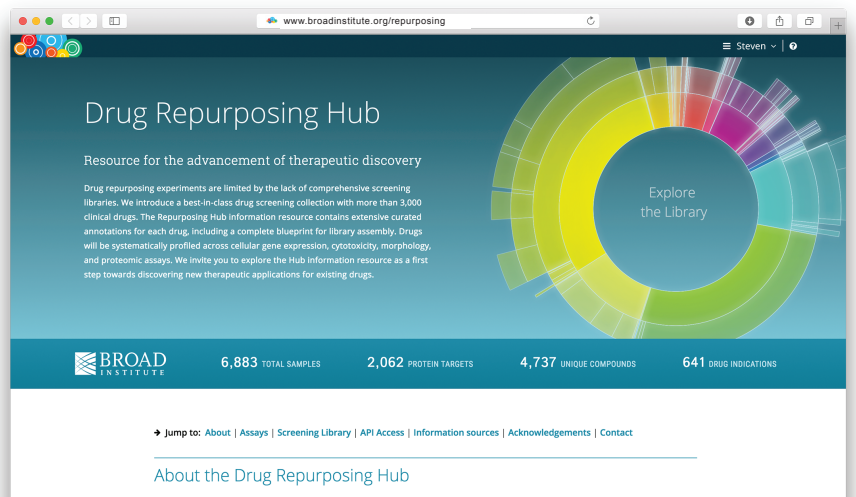
A) All compound samples were assessed by liquid chromatography-mass spectrometry to confirm compound identity and purity. In cases where multiple samples of the same compound were obtained, the best purity score for that compound is shown. Compounds were rejected if the purity was less than 85%. Each independent sample was tested once. B) Samples received in DMSO solution had lower purity. One chemical vendor re-supplied compounds with less than 90% purity upon initial testing of the DMSO samples (Batch 1). The second batch was shipped as dry powder (Batch 2).

Figure S5

Supplementary Figure 5: The Drug Repurposing Hub website.

A) The landing page of the Hub website displays updated library statistics. B) An interactive web application provides curated compound annotations, quality control results, and links to external databases. The Hub entry for sirolimus is shown as an example.

A



B

CP
< >

Sirolimus

☆

Sample 1 | 2 | 3

Broad ID: BRD-K84937637-001-09-9
PubChem CID: 5284616
Vendor: Selleck - Rapamycin (Sirolimus) (S1039)

Expected mass: 913.555
Purity: 95.47
QC result: PASS

Structure:
InChIKey: QFJCIRLUMZQUOT-HPLJQOBZSA-N
SMILES: CO[C@@H]1C[C@@H](C[C@@H](C)[C@@H]2CC(=O)[C@@H](C)\C=C(C)\C@@H(O)[C@@H](OC)C(=O)[C@@H](C)C[C@@H](C)\C=C-C=C-C(C)\C@@H(C)[C@@H]3CC[C@@H](C)[C@@H](O)(O3)C(=O)N3CCC[C@@H]3C(=O)O)CC[C@H]1O

Clinical Information
Clinical Status: Launched
FDA Orange Book: sirolimus
Drugs@FDA: rapamune, sirolimus

Indications:
[Organ rejection \(transplant\)](#)
[Lymphangioleiomyomatosis \(pulmonary\)](#)

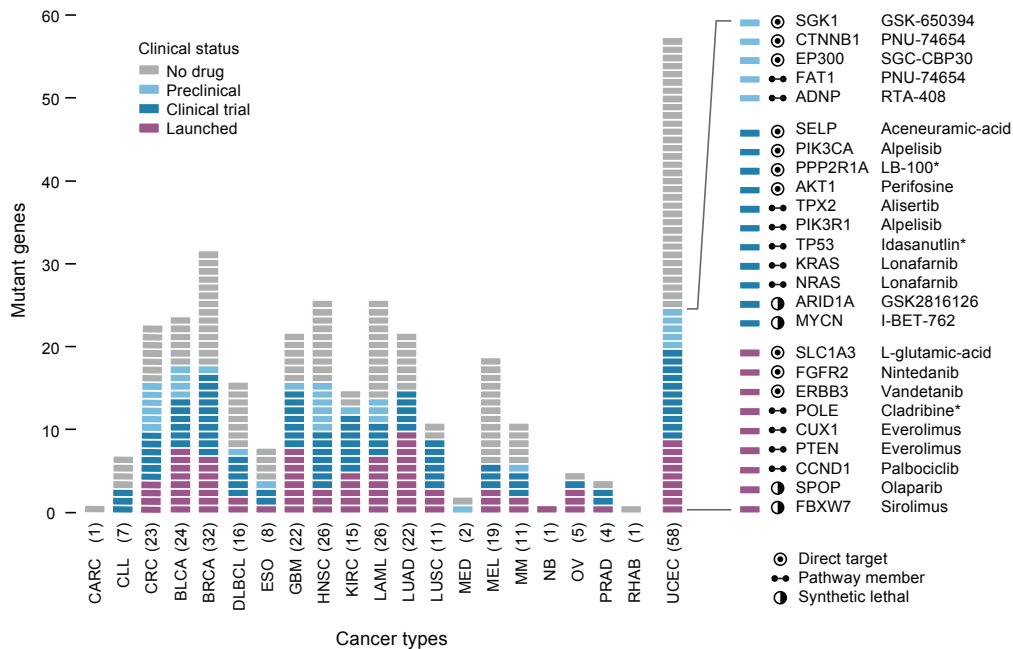
Mechanisms of Action
mTOR inhibitor

	1	2	3	4	Sources
MTOR	⊙	⊙	⊙	⊙	1 ChEMBL
FKBP1A	⊙	⊙	⊙	⊙	2 DrugBank
FGF2	⊙	⊙	⊙	⊙	3 IUPHAR
					4 TTD

Assay Data: [L1000](#) | [PRISM](#) | [Cell Painting](#) | [P100/GCP](#)

[Leave us a comment](#)

Figure S6



Supplementary Figure 6: Repurposing Hub drug coverage of significantly mutated genes in cancer. Tumor types from the Cancer Genome Atlas are listed in columns. For each statistically significantly mutated gene, the highest development status of a corresponding drug is indicated by a color code. Specific drug target information is shown for uterine corpus endometrial carcinoma (UCEC) as an example. Mechanisms of drug activity include direct targeting of the corresponding protein product, targeting of a downstream pathway member, or recapitulating a known synthetic lethal interaction. In some cases, marked by *, gene mutation leads to loss of protein function that is unlikely to be reversed by the indicated small molecule inhibitor.

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Supplementary Table 1: Notable drug repurposing discoveries.

Clinical use	
Aspirin to treat coronary artery disease	Flossmann, E., Rothwell, P. M. British Doctors Aspirin Trial and the UK-TIA Aspirin Trial. Effect of aspirin on long-term risk of colorectal cancer: consistent evidence from randomised and observational studies. <i>Lancet</i> 369 , 1603–1613 (2007).
Erythromycin to treat impaired gastric motility	Sturm, A., Holtmann, G., Goebell, H. & Gerken, G. Prokinetics in patients with gastroparesis: a systematic analysis. <i>Digestion</i> 60 , 422–427 (1999).
Sildenafil to treat erectile dysfunction	Goldstein, I. <i>et al.</i> Oral sildenafil in the treatment of erectile dysfunction. Sildenafil Study Group. <i>N. Engl. J. Med.</i> 338 , 1397–1404 (1998).
Thalidomide to treat multiple myeloma	Palumbo, A. <i>et al.</i> Thalidomide for treatment of multiple myeloma: 10 years later. <i>Blood</i> 111 , 3968–3977 (2008).
Gene expression discovery	
Imipramine to treat small cell lung cancer	Jahchan, N. S. <i>et al.</i> A drug repositioning approach identifies tricyclic antidepressants as inhibitors of small cell lung cancer and other neuroendocrine tumors. <i>Cancer Discovery</i> 3 , 1364–1377 (2013).
Sirolimus to treat glucocorticoid-resistant acute lymphocytic leukemia	Wei, G. <i>et al.</i> Gene expression-based chemical genomics identifies rapamycin as a modulator of MCL1 and glucocorticoid resistance. <i>Cancer Cell</i> 10 , 331–342 (2006).
Topiramate to treat inflammatory bowel disease	Dudley, J. T. <i>et al.</i> Computational repositioning of the anticonvulsant topiramate for inflammatory bowel disease. <i>Sci. Transl. Med.</i> 3 , 96ra76 (2011).
High-content imaging discovery	
Lovastatin to inhibit leukemia stem cells	Hartwell, K. A. <i>et al.</i> Niche-based screening identifies small-molecule inhibitors of leukemia stem cells. <i>Nat. Chem. Biol.</i> 9 , 840–848 (2013).

Supplementary Table 2: Additional resources relevant for drug repurposing experiments.

Cell line screening	
Analyzing activity data to determine molecular predictors of drug response	Basu, A. <i>et al.</i> An Interactive Resource to Identify Cancer Genetic and Lineage Dependencies Targeted by Small Molecules. <i>Cell</i> 154 , 1151–1161 (2013).
Molecular features of cell lines (e.g., gene expression, mutation, or copy number variation)	Barretina, J. <i>et al.</i> The Cancer Cell Line Encyclopedia enables predictive modelling of anticancer drug sensitivity. <i>Nature</i> 483 , 603–607 (2012).
Multiplex cytological imaging assay	Gustafsdottir, S. M. <i>et al.</i> Multiplex cytological profiling assay to measure diverse cellular states. <i>PLoS ONE</i> 8 , e80999 (2013).
Multiplex cytotoxicity profiling assay	Yu, C. <i>et al.</i> High-throughput identification of genotype-specific cancer vulnerabilities in mixtures of barcoded tumor cell lines. <i>Nat. Biotechnol.</i> 34 , 419–423 (2016).
Systematic gene expression profiling	Lamb, J. <i>et al.</i> The Connectivity Map: using gene-expression signatures to connect small molecules, genes, and disease. <i>Science</i> 313 , 1929–1935 (2006).
Intellectual property	
Relevance of drug patent and exclusivity status for repurposing	Smith, R. B. Repositioned drugs: integrating intellectual property and regulatory strategies. <i>Drug Discovery Today: Therapeutic Strategies</i> 8 , 131–137 (2011).
Metadata capture	
Published approach to capture experimental protocols using controlled vocabulary terms	Howe, E. A. <i>et al.</i> BioAssay Research Database (BARD): chemical biology and probe-development enabled by structured metadata and result types. <i>Nucleic Acids Res.</i> 43 , D1163–70 (2015).

Supplementary Table 3

Source	Approval status	Structures	URL
Proprietary databases			
CAS SciFinder®	N	Y	http://www.cas.org/products/scifinder/
Citeline Pharmaprojects®	Y	Y	https://citeline.com/products/pharmaprojects/
Thomson Reuters Cortellis™	Y	Y	http://thomsonreuters.com/en/products-services/pharma-life-sciences/pharma-competitive-intelligence/cortellis-competitive-intelligence.html
Thomson Reuters Integrity SM	Y	Y	http://thomsonreuters.com/en/products-services/pharma-life-sciences/pharmaceutical-research/integrity.html
Public databases			
ChEMBL	Y	Y	https://www.ebi.ac.uk/chembl/
ClinicalTrials.gov	Y	N	https://clinicaltrials.gov/
DrugBank (4.3)	Y	Y	http://www.drugbank.ca/
EU Clinical Trials Register	Y	N	https://www.clinicaltrialsregister.eu/
IUPHAR Guide to Pharmacology	Y	Y	http://www.guidetopharmacology.org/
NCATS NPC	Y	Y	http://tripod.nih.gov/npc/
Regulatory agencies			
Canada DPD	Y	N	http://www.hc-sc.gc.ca/dhp-mps/prodpharma/databasdon/dpd_bdpp_data_extract-eng.php
Drugs@FDA	Y	N	https://www.accessdata.fda.gov/scripts/cder/drugsatfda/
EMA Belgium	Y	N	http://www.fagg-afmps.be/nl/items/gegevensbank_vergunde_geneesmiddelen/
FDA Orange Book	Y	N	http://www.accessdata.fda.gov/scripts/cder/ob/
FDA OTC	Y	N	http://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/CDER/ucm106368.htm
Japan PMDA	Y	N	https://www.pmda.go.jp/english/review-services/reviews/approved-information/drugs/0002.html
NIH DailyMed	Y	N	https://dailymed.nlm.nih.gov/dailymed/index.cfm

Supplementary Table 3: Databases searched for clinical drug chemical structures and annotations.

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Supplementary Table 4: Repurposing Hub drug coverage of significantly mutated genes in cancer.

TCGA type	Mutant gene	Drug	Mechanism	Drug target	Drug status	Relationship source
BLCA	CREBBP	SGC-CBP30	direct target	CREBBP	Preclinical	IUPHAR
BLCA	ERBB3	vandetanib	direct target	ERBB3	Launched	ChEMBL
BLCA	FGFR3	masitinib	direct target	FGFR3	Launched	http://meetinglibrary.asco.org/content/30794-65
BLCA	KDM6A	GSK-J4	direct target	KDM6A	Preclinical	http://www.chemicalprobes.org/GSK-J4/overview
BLCA	NFE2L2	RTA-408	direct target	NFE2L2	Phase 2	https://en.wikipedia.org/wiki/NFE2L2
BLCA	PIK3CA	alpelisib	direct target	PIK3CA	Phase 3	IUPHAR
BLCA	RXRA	acitretin	direct target	RXRA	Launched	DrugBank
BLCA	CDKN1A	GGTI-298	pathway member	CDKN1A	Preclinical	PMID9341167
BLCA	FOXQ1	benzyl-isothiocyanate	pathway member		Preclinical	PMID23276794
BLCA	HRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
BLCA	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
BLCA	TSC1	sirolimus	pathway member	MTOR	Launched	PMID20048174
BLCA	ARID1A	GSK2816126	synthetic lethality	EZH2	Phase 1	PMID25686104
BLCA	ERCC2	cisplatin	synthetic lethality		Launched	PMID25096233
BLCA	FBXW7	sirolimus	synthetic lethality	MTOR	Launched	PMID18787170
BLCA	MLL2	EPZ-5676	synthetic lethality	DOT1L	Phase 1	PMID23649466
BLCA	RB1	etoposide	synthetic lethality	TOP2A	Launched	PMID9032231
BLCA	STAG2	olaparib	synthetic lethality	PARP1	Launched	PMID24356817
BRCA	AKT1	perifosine	direct target	AKT1	Phase 3	TTD
BRCA	ERBB2	afatinib	direct target	ERBB2	Launched	DrugBank; IUPHAR; TTD
BRCA	MAP2K4	genistein	direct target	MAP2K4	Phase 2/Phase 3	PMID25019290
BRCA	MAP3K1	PD-184352	direct target	MAP3K1	Phase 2	TTD
BRCA	MLL	MM-102	direct target	MLL	Preclinical	PMID23210835
BRCA	PIK3CA	alpelisib	direct target	PIK3CA	Phase 3	IUPHAR
BRCA	CASP8	PAC-1	pathway member	CASP3	Phase 1	PMID16936720
BRCA	CDH1	dinaciclib	pathway member	CDK5	Phase 3	PMID26658992
BRCA	CUL4B	thalidomide	pathway member	CRBN	Launched	PMID26460955
BRCA	GATA3	dexamethasone	pathway member	NR3C1	Launched	PMID19124555
BRCA	KRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
BRCA	PIK3R1	alpelisib	pathway member	PIK3CA	Phase 3	PMID18794884
BRCA	PTEN	everolimus	pathway member	MTOR	Launched	PMID23582881
BRCA	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
BRCA	ARID1A	GSK2816126	synthetic lethality	EZH2	Phase 1	PMID25686104
BRCA	RB1	etoposide	synthetic lethality	TOP2A	Launched	PMID9032231
BRCA	RUNX1	olaparib	synthetic lethality	PARP1	Launched	PMID26594843
BRCA	STAG2	olaparib	synthetic lethality	PARP1	Launched	PMID24356817
CLL	RPS2	emetine	direct target	RPS2	Phase 2	http://en.wikipedia.org/wiki/Emetine
CLL	XPO1	selinexor	direct target	XPO1	Phase 2/Phase 3	http://www.selleckchem.com/products/kpt-330.html
CLL	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
CRC	ACVR1B	SB-431542	direct target	ACVR1B	Preclinical	PMID12065756
CRC	B2M	3-indolebutyric-acid	direct target	B2M	Preclinical	DrugBank
CRC	BRAF	dabrafenib	direct target	BRAF	Launched	DrugBank; IUPHAR; TTD
CRC	ERBB3	vandetanib	direct target	ERBB3	Launched	ChEMBL

TCGA type	Mutant gene	Drug	Mechanism	Drug target	Drug status	Relationship source
CRC	GOT1	L-Aspartic-Acid	direct target	GOT1	Launched	DrugBank
CRC	PIK3CA	alpelisib	direct target	PIK3CA	Phase 3	IUPHAR
CRC	SMAD2	dexfosfoserine	direct target	SMAD2	Preclinical	DrugBank
CRC	APC	XAV-939	pathway member	TNKS1	Preclinical	PMID19759537
CRC	AXIN2	XAV-939	pathway member	TNKS1	Preclinical	PMID19759537
CRC	CASP8	PAC-1	pathway member	CASP3	Phase 1	PMID16936720
CRC	CDC27	TAME	pathway member	APC	Preclinical	PMID20951947
CRC	KRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
CRC	NRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
CRC	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
CRC	ARID1A	GSK2816126	synthetic lethality	EZH2	Phase 1	PMID25686104
CRC	FBXW7	sirolimus	synthetic lethality	MTOR	Launched	PMID18787170
DLBCL	BRAF	dabrafenib	direct target	BRAF	Launched	DrugBank; IUPHAR; TTD
DLBCL	CREBBP	SGC-CBP30	direct target	CREBBP	Preclinical	IUPHAR
DLBCL	EZH2	tazemetostat	direct target	EZH2	Phase 2	IUPHAR
DLBCL	TNF	VGX-1027	direct target	TNF	Phase 1	http://www.abcam.com/vgx-1027-git-27-ab145857.html
DLBCL	CARD11	mepazine	pathway member	MALT1	Phase 2	PMID23238017
DLBCL	CD79B	ibrutinib	pathway member	BTK	Launched	PMID26193343
DLBCL	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
DLBCL	MLL2	EPZ-5676	synthetic lethality	DOT1L	Phase 1	PMID23649466
ESO	PIK3CA	alpelisib	direct target	PIK3CA	Phase 3	IUPHAR
ESO	FLG	JTC-801	pathway member	OPRL1	Phase 2	PMID24055295
ESO	IL7R	ruxolitinib	pathway member	JAK2	Launched	PMID26208852
ESO	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
GBM	AZGP1	aceneuramic-acid	direct target	AZGP1	Phase 3	Drugbank
GBM	BRAF	dabrafenib	direct target	BRAF	Launched	DrugBank; IUPHAR; TTD
GBM	DDX5	resveratrol	direct target	DDX5	Launched	PMID27148684
GBM	EGFR	afatinib	direct target	EGFR	Launched	DrugBank; IUPHAR; TTD
GBM	IDH1	AGI-5198	direct target	IDH1	Preclinical	http://www.apexbt.com/agi-5198.html
GBM	MAP3K1	PD-184352	direct target	MAP3K1	Phase 2	TTD
GBM	PIK3CA	alpelisib	direct target	PIK3CA	Phase 3	IUPHAR
GBM	CD1D	vorinostat	pathway member	HDAC1	Launched	PMID22419072
GBM	PIK3R1	alpelisib	pathway member	PIK3CA	Phase 3	PMID18794884
GBM	PTEN	everolimus	pathway member	MTOR	Launched	PMID23582881
GBM	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
GBM	CHD8	I-BET-762	synthetic lethality	BRD4	Phase 2	PMID24584072
GBM	NF1	sirolimus	synthetic lethality	MTOR	Launched	PMID15937108
GBM	RB1	etoposide	synthetic lethality	TOP2A	Launched	PMID9032231
GBM	SETD2	AZD6482	synthetic lethality	PIK3CB	Phase 1	PMID26537074
GBM	STAG2	olaparib	synthetic lethality	PARP1	Launched	PMID24356817
HNSC	B2M	3-indolebutyric-acid	direct target	B2M	Preclinical	DrugBank
HNSC	EP300	SGC-CBP30	direct target	EP300	Preclinical	IUPHAR
HNSC	EPHA2	dasatinib	direct target	EPHA2	Launched	ChEMBL; DrugBank
HNSC	MAP4K3	bosutinib	direct target	MAP4K3	Launched	PMID22037378
HNSC	NFE2L2	RTA-408	direct target	NFE2L2	Phase 2	https://en.wikipedia.org/wiki/NFE2L2
HNSC	NOTCH1	FLI-06	direct target	NOTCH1	Preclinical	PMID24077179

TCGA type	Mutant gene	Drug	Mechanism	Drug target	Drug status	Relationship source
HNSC	PIK3CA	alpelisib	direct target	PIK3CA	Phase 3	IUPHAR
HNSC	TGFBR2	LY2109761	direct target	TGFBR2	Preclinical	IUPHAR
HNSC	AJUBA	PNU-74654	pathway member	CTNNB1	Preclinical	PMID17621269
HNSC	CASP8	PAC-1	pathway member	CASP3	Phase 1	PMID16936720
HNSC	FAT1	PNU-74654	pathway member	CTNNB1	Preclinical	PMID23354438
HNSC	HRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
HNSC	PTEN	everolimus	pathway member	MTOR	Launched	PMID23582881
HNSC	RHOA	Y-39983	pathway member	ROCK1	Phase 2	PMID25010901
HNSC	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
HNSC	MLL2	EPZ-5676	synthetic lethality	DOT1L	Phase 1	PMID23649466
KIRC	ATM	VE-822	direct target	ATM	Phase 2	IUPHAR
KIRC	GUSB	L-Aspartic-Acid	direct target	GUSB	Launched	PMID11568288
KIRC	KDM5C	IOX2	direct target	KDM5C	Preclinical	IUPHAR
KIRC	MPO	cefdinir	direct target	MPO	Launched	DrugBank
KIRC	MTOR	everolimus	direct target	MTOR	Launched	DrugBank; IUPHAR; TTD
KIRC	PTEN	everolimus	pathway member	MTOR	Launched	PMID23582881
KIRC	RHEB	lonafarnib	pathway member	FNTA	Phase 3	PMID16006564
KIRC	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
KIRC	ARID1A	GSK2816126	synthetic lethality	EZH2	Phase 1	PMID25686104
KIRC	BAP1	GSK2816126	synthetic lethality	EZH2	Phase 1	PMID26437366
KIRC	PBRM1	GSK2816126	synthetic lethality	EZH2	Phase 1	PMID26552009
KIRC	SETD2	AZD6482	synthetic lethality	PIK3CB	Phase 1	PMID26537074
KIRC	VHL	homoharringtonine	synthetic lethality	RPL3	Launched	PMID26219258
LAML	DNMT3A	azacitidine	direct target	DNMT3A	Launched	ChEMBL
LAML	EZH2	tazemetostat	direct target	EZH2	Phase 2	IUPHAR
LAML	FLT3	sorafenib	direct target	FLT3	Launched	DrugBank; IUPHAR
LAML	IDH1	AGI-5198	direct target	IDH1	Preclinical	http://www.apexbt.com/agi-5198.html
LAML	IDH2	AGI-6780	direct target	IDH2	Preclinical	http://www.apexbt.com/agi-6780.html
LAML	KIT	dasatinib	direct target	KIT	Launched	ChEMBL; DrugBank
LAML	PTPN11	BVT-948	direct target	PTPN11	Preclinical	http://www.abcam.com/bvt-948-ab141304.html
LAML	KRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
LAML	NRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
LAML	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
LAML	NPM1	tretinoin	synthetic lethality	RARA	Launched	PMID26022051
LAML	RAD21	olaparib	synthetic lethality	PARP1	Launched	PMID22412391
LAML	RUNX1	olaparib	synthetic lethality	PARP1	Launched	PMID26594843
LAML	STAG2	olaparib	synthetic lethality	PARP1	Launched	PMID24356817
LUAD	ATM	VE-822	direct target	ATM	Phase 2	IUPHAR
LUAD	BRAF	dabrafenib	direct target	BRAF	Launched	DrugBank; IUPHAR; TTD
LUAD	EGFR	afatinib	direct target	EGFR	Launched	DrugBank; IUPHAR; TTD
LUAD	ERBB2	afatinib	direct target	ERBB2	Launched	DrugBank; IUPHAR; TTD
LUAD	KEAP1	dimethyl-fumarate	direct target	KEAP1	Launched	ChEMBL; DrugBank
LUAD	MAP2K1	trametinib	direct target	MAP2K1	Launched	DrugBank; IUPHAR
LUAD	MET	alectinib	direct target	MET	Launched	IUPHAR
LUAD	PIK3CA	alpelisib	direct target	PIK3CA	Phase 3	IUPHAR
LUAD	STK11	nintedanib	direct target	STK11	Launched	PMID22037378

TCGA type	Mutant gene	Drug	Mechanism	Drug target	Drug status	Relationship source
LUAD	KRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
LUAD	RIT1	trametinib	pathway member	MAP2K1	Launched	PMID24469055
LUAD	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
LUAD	ARID1A	GSK2816126	synthetic lethality	EZH2	Phase 1	PMID25686104
LUAD	NF1	sirolimus	synthetic lethality	MTOR	Launched	PMID15937108
LUAD	RB1	etoposide	synthetic lethality	TOP2A	Launched	PMID9032231
LUSC	KEAP1	dimethyl-fumarate	direct target	KEAP1	Launched	ChEMBL ; DrugBank
LUSC	NFE2L2	RTA-408	direct target	NFE2L2	Phase 2	https://en.wikipedia.org/wiki/NFE2L2
LUSC	PIK3CA	alpelisib	direct target	PIK3CA	Phase 3	IUPHAR
LUSC	HRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
LUSC	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
LUSC	ARID1A	GSK2816126	synthetic lethality	EZH2	Phase 1	PMID25686104
LUSC	FBXW7	sirolimus	synthetic lethality	MTOR	Launched	PMID18787170
LUSC	MLL2	EPZ-5676	synthetic lethality	DOT1L	Phase 1	PMID23649466
LUSC	RB1	etoposide	synthetic lethality	TOP2A	Launched	PMID9032231
MED	CTNNB1	PNU-74654	direct target	CTNNB1	Preclinical	http://www.tocris.com/disprod.php?ItemId=225597
MEL	BRAF	dabrafenib	direct target	BRAF	Launched	DrugBank ; IUPHAR ; TTD
MEL	CDK4	palbociclib	direct target	CDK4	Launched	IUPHAR ; TTD
MEL	NRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
MEL	PTEN	everolimus	pathway member	MTOR	Launched	PMID23582881
MEL	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
MEL	PPP6C	alisertib	synthetic lethality	AURKA	Phase 3	PMID24336958
MM	BRAF	dabrafenib	direct target	BRAF	Launched	DrugBank ; IUPHAR ; TTD
MM	IDH1	AGI-5198	direct target	IDH1	Preclinical	http://www.apexbt.com/agi-5198.html
MM	IRF4	simvastatin	pathway member	HMGCR	Launched	PMID21856936
MM	KRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
MM	NRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
MM	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
NB	ALK	alectinib	direct target	ALK	Launched	IUPHAR
OV	CDK12	alvocidib	direct target	CDK12	Phase 2	PMID24662513
OV	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
OV	BRCA1	olaparib	synthetic lethality	PARP1	Launched	PMID25366685
OV	NF1	sirolimus	synthetic lethality	MTOR	Launched	PMID15937108
OV	RB1	etoposide	synthetic lethality	TOP2A	Launched	PMID9032231
PRAD	ATM	VE-822	direct target	ATM	Phase 2	IUPHAR
PRAD	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
PRAD	SPOP	olaparib	synthetic lethality	PARP1	Launched	PMID26374986
UCEC	AKT1	perifosine	direct target	AKT1	Phase 3	TTD
UCEC	CTNNB1	PNU-74654	direct target	CTNNB1	Preclinical	http://www.tocris.com/disprod.php?ItemId=225597
UCEC	EP300	SGC-CBP30	direct target	EP300	Preclinical	IUPHAR
UCEC	ERBB3	vandetanib	direct target	ERBB3	Launched	ChEMBL
UCEC	FGFR2	nintedanib	direct target	FGFR2	Launched	IUPHAR
UCEC	PIK3CA	alpelisib	direct target	PIK3CA	Phase 3	IUPHAR
UCEC	PPP2R1A	LB-100	direct target	PPP2R1A	Phase 1	PMID23780887
UCEC	SELP	aceneuramic-acid	direct target	SELP	Phase 3	DrugBank ; TTD
UCEC	SGK1	GSK-650394	direct target	SGK1	Preclinical	IUPHAR

TCGA type	Mutant gene	Drug	Mechanism	Drug target	Drug status	Relationship source
UCEC	SLC1A3	L-glutamic-acid	direct target	SLC1A3	Launched	DrugBank
UCEC	ADNP	RTA-408	pathway member	NFE2L2	Preclinical	PMID19130308
UCEC	CCDC6	P22077	pathway member	USP7	Preclinical	PMID27372520
UCEC	CCND1	palbociclib	pathway member	CDK4	Launched	PMID25524798
UCEC	CUX1	everolimus	pathway member	MTOR	Launched	PMID24316979
UCEC	FAT1	PNU-74654	pathway member	CTNNB1	Preclinical	PMID23354438
UCEC	KRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
UCEC	NRAS	lonafarnib	pathway member	FNTA	Phase 3	PMID25878363
UCEC	PIK3R1	alpelisib	pathway member	PIK3CA	Phase 3	PMID18794884
UCEC	POLE	cladribine	pathway member	ADA	Launched	PMID18794884
UCEC	PTEN	everolimus	pathway member	MTOR	Launched	PMID23582881
UCEC	TP53	idasanutlin	pathway member	MDM2	Phase 3	PMID24188661
UCEC	TPX2	alisertib	pathway member	AURKA	Phase 3	PMID21879811
UCEC	ARID1A	GSK2816126	synthetic lethality	EZH2	Phase 1	PMID25686104
UCEC	FBXW7	sirolimus	synthetic lethality	MTOR	Launched	PMID18787170
UCEC	MYCN	I-BET-762	synthetic lethality	BRD4	Phase 2	PMID23430699
UCEC	SPOP	olaparib	synthetic lethality	PARP1	Launched	PMID26374986