**tool distribution program: cftr compound ordering and registration**

**program description**

The Chemical Compound Distribution Program is sponsored by CFF and administered by Professor Robert Bridges’ lab at Rosalind Franklin University of Science and Medicine. The program’s main purpose is to enable CF research scientists to test known CFTR modulating compounds in different functional assays. In return, scientists are expected to share their experiences in working with the compounds for the general benefit of the community and in service of CFF’s mission to find the means to cure and control cystic fibrosis.

**process overview**

1. The first step of the process is to have a *Material Transfer Agreement* in place between the ordering institution and the Rosalind Franklin University. Please send a completed and signed copy of the MTA to Kim Hankin at [Kim.Hankin@rosalindfranklin.edu](mailto:Kim.Hankin@rosalindfranklin.edu). A copy of the MTA can be obtained on the cff.org website in the For Researchers section under Antibodies and Modulators or can be requested from Kim Hankin.
2. Once an MTA is in place please forward a completed Order Form (Appendix A) to Kim Hankin. A completed order form must have the following:
   1. A correct address for shipments, most shipping companies will not deliver to PO Boxes.
   2. Shipping account number as Investigators will be responsible for all shipping costs associated with their orders
3. All completed orders received by 12:00 pm Eastern, Thursday will be processed and shipped within two weeks, depending on availability. Incomplete orders may incur a delay.

***Please Note:***

In any correspondence please list the requesting institution as well as investigator; this will help keep track of the various orders, especially if correspondence originates from multiple departments in the requesting intuition.

Order fulfillment is subject to approval by CFF and approved orders are filled on a first come, first serve basis.

Reorders of dry powder molecules within 12 months will be subject to CFF approval. *Note, 50 mg is a very large amount of material, and recipients are expected to conserve the reagent so that the supply will last over several years.*

**reporting**

Once every six months, you may be asked to complete a chemical compound report. CFF reserves the right to post information from these reports in a public web site describing these compounds.

**appendix a: chemical compound order form**

**compound ordering options, quantity and requirements**

You may select EITHER Option A *OR* Option B:

**Option A:**

250 microliters of a 4 mg/ml stock

Up to 10 compounds may be selected

**Option B:**

50 mg of dry powder

Up to 6 compounds may be selected

Additional numbers of compounds, volume or amount is subject to CFF approval and you may be asked for further justification.

**By submitting this application packet, the company, institution or investigator agrees to the following:**

* That compound orders are subject to approval by CFF and approved orders are filled on a first come, first served basis.
* That depending upon demand, compound availabilitymay be limited in which case CFF will suggest substitutions or you may be asked to wait until additional compound has been synthesized.
* That CFF makes no claims regarding these compounds, including the utility or fitness for a particular purpose.
* Requesting parties acknowledge Robert Bridges Ph.D., Rosalind Franklin University of Medicine and Science, and Cystic Fibrosis Foundation within each manuscript or public presentations demonstrating data generated using the compounds received through this program. Please send a copy of each manuscript to Christopher Penland, Ph.D. at [cpenland@cff.org](mailto:cpenland@cff.org).

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| **Order Form** | Please fill out all of the fields below (you may append additional information) |
| **Company or Institution:** |  |
| **Principal Investigator:** |  |
| **Primary Contact:** |  |
| **Contact Phone Number:** |  |
| **Contact Fax Number:** |  |
| **Contact Email:** |  |
| **Shipping Address:** |  |
| **Billing Address:** |  |
| **FedEx Shipping Number:** |  |
| **Description of Research Plan including which Cell Lines/Types are to be tested:** |  |
| **If this is a reorder include further description and justification as an addendum to original request:** |  |

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| **Option 1: DMSO Compound Order**  *250 microliters of a 4 mg/ml stock* | |  | **Option 2: Dry Powder**  *50 mg of dry powder* | |
| **ID** | **Amount** |  | **ID** | **Amount** |
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**appendix b: chemical compound inventory listing**

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| **ID** | **Chemical  Name** | **Chemical Structure** | **Comments** |
| **Blocker (B); Potentiator (P); Corrector (C); NMD-Inhibitor (N)** | | |  |
| **B1** | 4-[4-Oxo-2-thioxo-3-(3-trifluoromethyl-phenyl)-thiazolidin-5-ylidenemethyl]-benzoic acid |  | **Reference #\*\* 1 Name: CFinh-172** Potency:Ki= 300 nM Solvent:DMSO Hints For Use: Slow onset of inhibition in some cell types (e.g. T84 cells) requiring prolonged incubation.  M.W.: 409 |
| **B2** | (Naphthalen-2-ylamino)-acetic acid (3,5-dibromo-2,4,-dihydroxy-benzylidene)-hydrazide |  | **Reference #\*\* 2 Name: GlyH-101** Potency:Ki= 5 microM Solvent:DMSO Hints For Use: M.W.: 493 |
| **B3** | Diarylsulfonylurea |  | **Reference #\*\* 3 Name: DASU-01** Potency:Ki > 100 microM Solvent: Water or buffer Hints For Use: Useful for CFTR noise analysis  M.W. 335.3 |
| **B4** | (7R,9S)-7,8-dihydroxy-3-(4-hydroxy-5-(hydroxymethyl)tetrahydrofuran-2-yl)-7,9-dimethyl-3,7,8,9-tetrahydropyrimido[1,2-i]purine-9-carboxylic acid |  | **Reference #\*\*16 Name: Blocker 5ab** Potency:Ki < 100 pM but see Ref.#17 Solvent: Water or buffer Hints For Use:  M.W.395.37 |
| **B5** | (2S,4R)-3,4-dihydroxy-2,4-dimethyl-3,4-dihydro-2H-pyrimido[2,1-a]isoquinoline-2-carboxylic acid |  | **Reference #\*\* 16 Name: Blocker 8ab** Potency:Ki < 20 nM but see Ref.# 17 Solvent: Water or buffer Hints For Use:  M.W. 288.3 |
| **B6** | 7,9-dimethyl-11-phenyl-6-(5-methylfuran-2-yl)-5,6-dihydro-pyrimido-[4’,5’-3,4]pyrrolo[1,2-*a]quinoxaline-8,10-(7H,9H)-dione* |  | **Reference # 22**  **Name: PPQ-102**.  Potency Ki=90 nM  Solvent: DMSO  Hints for use:  M.W. 438.48 |
| **B7** | 5-[[4-(2h-tetrazol-5-yl)phenyl]methylene]-2-thioxo-3-[3-(trifluoromethyl)phenyl]-4-thiazolidinone |  | **Reference # 23**  **Name: Tetrazolo–Inh.-172.**  Potency: Ki~1 microM  Solvent: DMSO  Hints for use: Reported to be more water soluble than Inh.-172  M.W. 433.43 |
| **B8** | 4-[[3-[3-(trifluoromethyl)phenyl]-2,4-dioxo-5-thiazolidinylidene] methyl]benzoic acid |  | **Reference # 23**  **Name: Oxo–Inh.-172.**  Potency: Ki~1 microM  Solvent: DMSO  Hints for use: Reported to be more water soluble than Inh.-172  M.W. 393.34 |
| **P1** | 4-Methyl-2-(5-phenyl-1H-pyrazol-3-yl)-phenol |  | **Reference #\*\*12 & 15 Name: VRT-532** Potency:Ks 3 to 5 microM Solvent:DMSO Hints For Use:  M.W.: 250 |
| **P2** | 2-[(2-1H-Indol-3-yl-acetyl)-methyl-amino]-N-(4-isopropyl-phenyl)-2-phenyl-acetamide |  | **Reference #\*\* 4 Name: PG-01** Potency:Ks= 300 nM Solvent:DMSO Hints For Use: M.W.:439.5 |
| **P3** | 6-(Ethyl-phenyl-sulfonyl)-4-oxo-1,4-dihydro-quinoline-3-carboxylic acid 2-methoxy-benzylamide |  | **Reference #\*\* 4 Name: SF-03** Potency:Ks= 30 nM Solvent:DMSO Hints For Use:  M.W.:491.6 |
| **P4** | 1-(3-chlorophenyl)-5-trifluoromethyl-3-hydrobenzimidazol-2-one |  | **Reference #\*\* 5 Name: UCCF-853** Potency:Ks= 3 microM Solvent:DMSO Hints For Use:  M.W.: 312.7 |
| **P5** | 2-(2-Chloro-benzoylamino)-4,5,6,7-tetrahydro-benzo[b]thiophene-3-carboxylic acid amide |  | **Reference #\*\* 6 Name: dF508**act**-02** Potency:Ks= 70 nM Solvent:DMSO Hints For Use:  M.W.: 334.8 |
| **P6** | 5,7,Dihydroxy-3-(4-hydroxy-phenyl)-chroman-4-one |  | **Reference #\*\* 8 Name: Genistein (discontinued - available from Sigma #G6649)** Potency:Ks= 10 to 30 microM Solvent:DMSO Hints For Use:  M.W.:272.3 |
| **P7** | 1-(5-Chloro-2-hydroxy-phenyl)-5-trifluoromethyl-1,3-dihydro-indol-2-one |  | **Reference #\*\* 8 Name: NSOO4** Potency:EC50 3 microM Solvent:DMSO Hints For Use: Does not work in excised patches.  M.W.: 327.7 |
| **P8** | 4-(4-Oxo-4H-benzo[h]chromen-2-yl)-pyridinium; bisulfate |  | **Reference #\*\* 9 and 10** Potency:Ks= 2 microM Solvent:DMSO Hints For Use: M.W.:371.4 |
| **P9** | 3-But-3-ynyl-5-methoxy-1-phenyl-1H-pyrazole-4-carbaldehyde |  | **Reference #\*\* 10** Potency:Ks= 10 microM Solvent:DMSO Hints For Use:  M.W.:254.3 |
| **P10** | 3-(2-Benzyloxy-phenyl)-5-chloromethyl-isoxazole |  | **Reference #\*\* 10** Potency:Ks > 50 microM Solvent:DMSO Hints For Use:  M.W.:299.8 |
| **C1** | 6-(1H-Benzoimidazol-2-ylsulfanylmethyl)-2-(6-methoxy-4-methyl-quinazolin-2-ylamino)-pyrimidin-4-ol |  | **Reference #\*\* 11** Potency:Ks= 3 microM Solvent:DMSO Hints For Use:  M.W.:445.5 |
| **C2** | 2-{1-[4-(4-Chloro-benzensulfonyl)-piperazin-1-yl]-ethyl}-4-piperidin-1-yl-quinazoline |  | **Reference. Vertex Presentation Name: VRT-640** Potency: unknown Solvent:DMSO Hints For Use: Likely binds to serum proteins.  M.W.:500.1 |
| **C3** | 4-Cyclohexyloxy-2-{1-[4-(4-methoxy-benzensulfonyl)-piperazin-1-yl]-ethyl}-quinazoline |  | **Reference #\*\* 12,13, 15 Name: VRT-325** Potency:EC50 2 microM Solvent: dry DMSO Hints For Use: Binds to serum proteins  M.W.:510.65 |
| **C4** | N-[2-(5-Chloro-2-methoxy-phenylamino)-4'-methyl-[4,5']bithiazolyl-2'-yl]-benzamide |  | **Reference #\*\* 11 Name: cmpd 4a** Potency:EC50 2 microM Solvent: DMSO Hints For Use:  M.W.:440.9 |
| **C5** | 4,5,7-trimethyl-N-phenylquinolin-2-amine |  | **Reference #\*\* 11: Name: cmpd 5a** Potency:EC50 13 microM Solvent: DMSO Hints For Use:  M.W.:262.35 |
| **C6** | N-(4-bromophenyl)-4-methylquinolin-2-amine |  | **Reference #\*\* 11: Name: cmpd 5c** Potency:EC50 8 microM Solvent: DMSO Hints For Use:  M.W.:313.19 |
| **C7** | 2-(4-isopropoxypicolinoyl)-N-(4-pentylphenyl)-1,2,3,4-tetrahydroisoquinoline-3-carboxamide |  | **Reference #\*\* 21: Name:Genzyme cmpd 48 only 10mg will be provided** Potency:EC50 300 nM Solvent: DMSO Hints For Use:  M.W.472.6 |
| **C8** | N-(2-fluorophenyl)-2-(1H-indol-3-yl)-2-oxoacetamide |  | **Reference #\*\* Vertex patent** Potency:EC50  Solvent:DMSO Hints For Use:  M.W.:282.27 |
| **C9** | 7-chloro-4-(4-(4-chlorophenylsulfonyl)piperazin-1-yl)quinoline |  | **Reference #\*\*18 Name: KM11060** Potency:EC50 < 1 microM Solvent:DMSO Hints For Use:  M.W.:422.33 |
| **C10** | 7-chloro-4-(4-(phenylsulfonyl)piperazin-1-yl)quinoline |  | **Reference # 18 Name: KM11057** Potency:EC50 > 100 microM Solvent:DMSO Hints For Use: Inactive derivative of C9 (KM11060)  M.W.:387.88 |
| **C11** | (Z)-N'-(3,4-dihydroxybenzylidene)-3-hydroxy-2-naphthohydrazide |  | **Reference #: 19 Name: Dynasore** Potency:EC50 10-20 microM Solvent: DMSO Hints For Use: An inhibitor of dynamin, blocks CFTR endocytosis  M.W.:322.31 |
| **C12** | N-(4-fluorophenyl)-4-p-tolylthiazol-2-amine | |  | | --- | |  | | **Reference #: 11 Name: 2i** Potency:EC50 5 microM Solvent: DMSO Hints For   M.W.:284.35 |
| **C13** | N-(2-(3-acetylphenylamino)-4'-methyl-4,5'-bithiazol-2'-yl)benzamide |  | **Reference #: 11 Name: 4c** Potency:EC50 2 microM Solvent: DMSO Hints For   M.W.:434.53 |
| **C14** | N-(2'-(2-methoxyphenylamino)-4-methyl-5,5'-bithiazol-2-yl)benzamide |  | **Reference #: 11 Name:4d** Potency:EC50 7 microM Solvent: DMSO Hints For Use:   M.W.422.52 |
| **C15** | N-phenyl-4-(4-vinylphenyl)thiazol-2-amine |  | **Reference #: 11 Name: 2b** Potency:EC50 16 microM Solvent: DMSO Hints   M.W.278.37 |
| **C16** | 2-(6-methoxy-4-methylquinazolin-2-ylamino)-5,6-dimethylpyrimidin-4(1H)-one |  | **Reference #: 11 Name: 3d** Potency:EC50 15 microM Solvent: DMSO Hints   M.W.:311.34 |
| **C17** | N-(2-(5-chloro-2-methoxyphenylamino)-4'-methyl-4,5'-bithiazol-2'-yl)pivalamide |  | **Reference #: 20 Name: 15Jf** Potency:EC50 1-2 microM Solvent: DMSO Hints For Use:   M.W.:436.98 |
| **C18** | 1-(benzo[d][1,3]dioxol-5-yl)-N-(5-((S)-(2-chlorophenyl)((R)-3-hydroxypyrrolidin-1-yl)methyl)thiazol-2-yl)cyclopropanecarboxamide | cid:_4_018CDC54018CD9E800576B3688257A1B | **Reference # 24**  **Name: VRT-534 (also known as CF-106951)**  **only 10 mg will be provided**  Potency Ks~0.6 microM  Solvent: DMSO  Hints for use: Use at 3 to 6 microM for maximum effect.  M.W. 497.99 |
|  | 6 compounds from the EPIX Pharmaceuticals Dual Corrector/Potentiator series are available through a specific MTA. | Please contact Kathryn Fox ([kfox@cff.org](mailto:kfox@cff.org)) to receive the MTA template. |  |
| **N1** | 2-chloro-N,N-diethyl-5-((4-(2-(4- (3-methylureido)phenyl)pyridin- 4-yl)pyrimidin-2-yl)  amino)benzenesulfonamide |  | **Reference # 25**  **Named SMG1i by CFFT Lab**  Potency: IC50 110 picoM  for purified enzyme Solvent: DMSO Hints For Use: 0.1–1 microM in cell-based assays M.W.: 566.07 |

*The order form is an open and working listing of available compounds. It is subject to change.*

# CFFT USE ONLY

APPROVED: \_\_\_\_\_\_\_\_\_\_\_

DATE: \_\_\_\_\_\_\_\_\_\_\_\_\_\_\_\_

ORDER #: \_\_\_\_\_\_\_\_\_\_\_\_\_

*All compounds are at least 95% pure; NMR and melting point data are available upon request.*

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