

(12) INTERNATIONAL APPLICATION PUBLISHED UNDER THE PATENT COOPERATION TREATY (PCT)

(19) World Intellectual Property
Organization
International Bureau

(43) International Publication Date
08 December 2022 (08.12.2022)



(10) International Publication Number
WO 2022/256642 A2

(51) International Patent Classification:
Not classified

(21) International Application Number:
PCT/US2022/032144

(22) International Filing Date:
03 June 2022 (03.06.2022)

(25) Filing Language: English

(26) Publication Language: English

(30) Priority Data:
63/197,073 04 June 2021 (04.06.2021) US
63/225,046 23 July 2021 (23.07.2021) US
63/292,889 22 December 2021 (22.12.2021) US
63/300,727 19 January 2022 (19.01.2022) US

(71) Applicant: **ARBOR BIOTECHNOLOGIES, INC.**
[US/US]; 20 Acorn Park Drive, Suite 500, Cambridge,
Massachusetts 02140 (US).

(72) Inventors: **WESSELLS, Quinton Norman**; c/o Arbor
Biotechnologies, Inc., 20 Acorn Park Drive, Suite 500,
Cambridge, Massachusetts 02140 (US). **HASWELL, Jef-
frey Raymond**; c/o Arbor Biotechnologies, Inc., 20 Acorn
Park Drive, Suite 500, Cambridge, Massachusetts 02140
(US). **DITOMMASO, Tia Marie**; c/o Arbor Biotechnolo-
gies, Inc., 20 Acorn Park Drive, Suite 500, Cambridge,

Massachusetts 02140 (US). **JAKIMO, Noah Michael**; c/
o Arbor Biotechnologies, Inc., 20 Acorn Park Drive, Suite
500, Cambridge, Massachusetts 02140 (US). **SENGUPTA,
Sejuti**; c/o Arbor Biotechnologies, Inc., 20 Acorn Park Drive,
Suite 500, Cambridge, Massachusetts 02140 (US).

(74) Agent: **DAVIS, J. Wendy**; THE MCDERMOTT
BUILDING, 500 NORTH CAPITOL STREET, N.W.,
Washington, District of Columbia 20001 (US).

(81) Designated States (unless otherwise indicated, for every
kind of national protection available): AE, AG, AL, AM,
AO, AT, AU, AZ, BA, BB, BG, BH, BN, BR, BW, BY, BZ,
CA, CH, CL, CN, CO, CR, CU, CZ, DE, DJ, DK, DM, DO,
DZ, EC, EE, EG, ES, FI, GB, GD, GE, GH, GM, GT, HN,
HR, HU, ID, IL, IN, IQ, IR, IS, IT, JM, JO, JP, KE, KG, KH,
KN, KP, KR, KW, KZ, LA, LC, LK, LR, LS, LU, LY, MA,
MD, ME, MG, MK, MN, MW, MX, MY, MZ, NA, NG, NI,
NO, NZ, OM, PA, PE, PG, PH, PL, PT, QA, RO, RS, RU,
RW, SA, SC, SD, SE, SG, SK, SL, ST, SV, SY, TH, TJ, TM,
TN, TR, TT, TZ, UA, UG, US, UZ, VC, VN, WS, ZA, ZM,
ZW.

(84) Designated States (unless otherwise indicated, for every
kind of regional protection available): ARIPO (BW, GH,
GM, KE, LR, LS, MW, MZ, NA, RW, SD, SL, ST, SZ, TZ,
UG, ZM, ZW), Eurasian (AM, AZ, BY, KG, KZ, RU, TJ,
TM), European (AL, AT, BE, BG, CH, CY, CZ, DE, DK,

(54) Title: GENE EDITING SYSTEMS COMPRISING AN RNA GUIDE TARGETING HYDROXYACID OXIDASE 1 (HAO1)
AND USES THEREOF

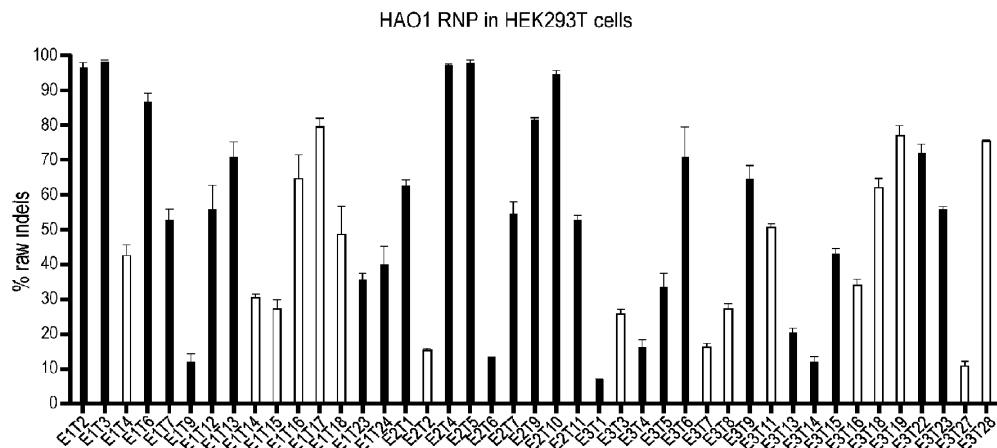


FIG. 1

(57) Abstract: Provided herein are gene editing systems and/or compositions comprising RNA guides targeting HAO1 for use in genetic editing of the HAO1 gene. Also provide herein are methods of using the gene editing system for introducing edits to the HAO1 gene and/or for treatment of primary hyperoxaluria (PH), and processes for characterizing the gene editing system.

[Continued on next page]

EE, ES, FI, FR, GB, GR, HR, HU, IE, IS, IT, LT, LU, LV,
MC, MK, MT, NL, NO, PL, PT, RO, RS, SE, SI, SK, SM,
TR), OAPI (BF, BJ, CF, CG, CI, CM, GA, GN, GQ, GW,
KM, ML, MR, NE, SN, TD, TG).

Published:

- *without international search report and to be republished
upon receipt of that report (Rule 48.2(g))*
- *with sequence listing part of description (Rule 5.2(a))*