

List of allowed mutations for Allele 2 (Heterozygote)

**Patients with F508del or other CFTR mutations (such as S549N and P205S or any other mutation; shown in bold font in the table below) responsive to Trikafta will be excluded from Treatment Period 6 and possibly Treatment Period 7.**

Variant (legacy)	Variant (cDNA)	Mutation Type	Class	Reference
1717-8G->A	c.1585-8G>A	Splicing	<b>1</b>	2
1811+1634A->G	c.1679+1634A>G	Splicing	<b>1</b>	3
1811+1643G->T	c.1680-877G>T	Splicing	<b>1</b>	4
1898+3A->G	c.1766+3A>G	Splicing	<b>1</b>	5
1898+5G->T	c.1766+5G>T	Splicing	<b>1</b>	6
3120G->A	c.2988G>A	Missense	<b>1</b>	2
3849+4A->G	c.3717+4A>G	Splicing	<b>1</b>	7
405+3A->C	c.273+3A>C	Splicing	<b>1</b>	5
R1066C	c.3196C>T	Missense	<b>1</b>	7
1078delT	c.948delT	Deletion	<b>1</b>	2
1154insTC	c.1021_1022dupTC	Insertion	<b>1</b>	2
1525-2A->G	c.1393-2A>G	Splicing	<b>1</b>	7
1717-1G->A	c.1585-1G>A	Splicing	<b>1</b>	8
1898+1G->A	c.1766+1G>A	Splicing	<b>1</b>	2
2184delA	c.2052delA	Deletion	<b>1</b>	2
2184insA	c.2052dupA	Insertion	<b>1</b>	9
3007delG	c.2875delG	Deletion	<b>1</b>	2
3120+1G->A	c.2988+1G>A	Splicing	<b>1</b>	2
3659delC	c.3528delC	Deletion	<b>1</b>	2
3876delA	c.3744delA	Deletion	<b>1</b>	2
3905insT	c.3773dupT	Insertion	<b>1</b>	1
4010del4	c.3883_3886delATTT	Deletion	<b>1</b>	2
4016insT	c.3884-3885insT	Insertion	<b>1</b>	2
4326delTC	c.4197_4198delCT	Deletion	<b>1</b>	2
4374+1G->T	c.4242+1G>T	Splicing	<b>1</b>	2
621+1G->T	c.489+1G>T	Splicing	<b>1</b>	1
711+1G->T	c.579+1G>T	Splicing	<b>1</b>	2
E1104X	c.3310G>T	Nonsense	<b>1</b>	2
E1371X	c.4111G>T	Nonsense	<b>1</b>	10
E193X	c.577G>T	Nonsense	<b>1</b>	10
E585X	c.1753G>T	Nonsense	<b>1</b>	2
E60X	c.178G>T	Nonsense	<b>1</b>	2
E822X	c.2464G>T	Nonsense	<b>1</b>	7
E92X	c.274G>T	Nonsense	<b>1</b>	10
G27X	c.79G>T	Nonsense	<b>1</b>	10
G330X	c.988G>T	Nonsense	<b>1</b>	10

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G542X	c.1624G>T	Nonsense	<b>1</b>	<i>1</i>
G550X	c.1648G>T	Nonsense	<b>1</b>	<i>10</i>
G673X	c.2017G>T	Nonsense	<b>1</b>	<i>10</i>
K710X	c.2128A>T	Nonsense	<b>1</b>	<i>10</i>
L1254X	c.3761T>G	Nonsense	<b>1</b>	<i>10</i>
L732X	c.2195T>G	Nonsense	<b>1</b>	<i>10</i>
L88X	c.263T>A or c.263T>G	Nonsense	<b>1</b>	<i>10</i>
Q1042X	c.3124C>T	Nonsense	<b>1</b>	<i>10</i>
Q1313X	c.3937C>T	Nonsense	<b>1</b>	<i>10</i>
Q1330X	c.3988C>T	Nonsense	<b>1</b>	<i>10</i>
Q1382X	c.4144C>T	Nonsense	<b>1</b>	<i>10</i>
Q1411X	c.4231C>T	Nonsense	<b>1</b>	<i>10</i>
Q1412X	c.4234C>T	Nonsense	<b>1</b>	<i>10</i>
Q220X	c.658C>T	Nonsense	<b>1</b>	<i>10</i>
Q2X	c.4C>T	Nonsense	<b>1</b>	<i>10</i>
Q30X	c.88C>T	Nonsense	<b>1</b>	<i>10</i>
Q39X	c.115C>T	Nonsense	<b>1</b>	<i>10</i>
Q414X	c.1240C>T	Nonsense	<b>1</b>	<i>10</i>
Q493X	c.1477C>T	Nonsense	<b>1</b>	<i>10</i>
Q525X	c.1573C>T	Nonsense	<b>1</b>	<i>10</i>
Q552X	c.1654C>T	Nonsense	<b>1</b>	<i>10</i>
Q685X	c.2053C>T	Nonsense	<b>1</b>	<i>10</i>
Q715X	c.2143C>T	Nonsense	<b>1</b>	<i>10</i>
Q720X	c.2158C>T	Nonsense	<b>1</b>	<i>10</i>
Q890X	c.2668C>T	Nonsense	<b>1</b>	<i>10</i>
Q98X	c.292C>T	Nonsense	<b>1</b>	<i>10</i>
R1102X	c.3304A>T	Nonsense	<b>1</b>	<i>10</i>
R1158X	c.3472C>T	Nonsense	<b>1</b>	<i>10</i>
R1162X	c.3484C>T	Nonsense	<b>1</b>	<i>1</i>
R553X	c.1657C>T	Nonsense	<b>1</b>	<i>2</i>
R709X	c.2125C>T	Nonsense	<b>1</b>	<i>10</i>
R75X	c.223C>T	Nonsense	<b>1</b>	<i>10</i>
R764X	c.2290C>T	Nonsense	<b>1</b>	<i>10</i>
R785X	c.2353C>T	Nonsense	<b>1</b>	<i>10</i>
R792X	c.2374C>T	Nonsense	<b>1</b>	<i>10</i>
R851X	c.2551C>T	Nonsense	<b>1</b>	<i>10</i>
S1196X	c.3587C>G	Nonsense	<b>1</b>	<i>10</i>
S1255X	c.3764C>A	Nonsense	<b>1</b>	<i>10</i>

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Variant (legacy)	Variant (cDNA)	Mutation Type	Class	Reference
S466X	c.1397C>A or c.1397C>G	Nonsense	1	10
S489X	c.1466C>A	Nonsense	1	10
S4X	c.11C>A	Nonsense	1	10
S912X	c.2735C>A	Nonsense	1	10
W1089X	c.3266G>A	Nonsense	1	10
W1098X	c.3293G>A or c.3294G>A	Nonsense	1	10
W1145X	c.3435G>A	Nonsense	1	10
W1204X	c.3611G>A or c.3612G>A	Nonsense	1	10
W1282X	c.3846G>A	Nonsense	1	2
W19X	c.57G>A	Nonsense	1	10
W216X	c.647G>A	Nonsense	1	10
W401X	c.1202G>A or c.1203G>A	Nonsense	1	10
W496X	c.1487G>A	Nonsense	1	10
W57X	c.170G>A or c.171G>A	Nonsense	1	10
W846X	c.2537G>A or c.2538G>A	Nonsense	1	10
W882X	c.2645G>A	Nonsense	1	10
Y1092X	c.3276C>A or c.3276C>G	Nonsense	1	2
Y122X	c.366T>A	Nonsense	1	10
Y275X	c.825C>G	Nonsense	1	10
Y849X	c.2547C>A	Nonsense	1	10
Y913X	c.2739T>A	Nonsense	1	10
<b>F508del</b>	c.1521_1523delCTT, or c.1521_1523del	Deletion	2	1
A559T	c.1675G>A	Missense	2	1
A561E	c.1682C>A	Missense	2	11
Y569D	c.1705T>G	Missense	2	12
L927P	c.2780T>C	Missense	2	12
R560T	c.1679G>C	Missense	2	2
S549R	c.1645A>C or c.1647T>G or c.1647T>A	Missense	2	1
E92K	c.274G>A	Missense	2	13
I507del	c.1519_1521delATC	Deletion	2	1
V520F	c.1558G>T	Missense	2	13
N1303K	c.3909C>G	Missense	2	1
M1V	c.1A>G	Missense	2	13
R334W	c.1000C>T	Missense	2	13
<b>S549N</b>	c.1646G>A	Missense	2	1
<b>P205S</b>	c.613C>T	Missense	2	13