**Supplementary Appendix**

**Appendix 1 – Full details of search strategy**

*Cystic Fibrosis AND:*

**Country names**

*Malta, Estonia, Finland, Bosnia and Herzegovina, Mexico, Guatemala, Cuba, Haiti, Dominican Republic, Honduras, Nicaragua, El Salvador, Costa Rica, Panama, Jamaica, Trinidad, India, China, Indonesia, Pakistan, Bangladesh, Japan, Philippines, Vietnam, Thailand, Myanmar, South Korea, North Korea, Uzbekistan, Malaysia, Nepal, Sri Lanka, Kazakhstan, Cambodia, Azerbaijan, Tajikistan, Laos, Kyrgyzstan, Turkmenistan, Singapore, Mongolia, Papua New Guinea, Oman, Qatar, Bahrain, Saudi Arabia, Iran, Jordan, Palestine, Lebanon, Syria, United Arab Emirates, Kuwait, Iraq, Afghanistan, Yemen, Chile, Argentina, Colombia, Peru, Venezuela, Ecuador, Bolivia, Paraguay, Uruguay, Egypt, Algeria, Libya, Sudan, Morocco, Nigeria, Ethiopia, Democratic Republic of Congo, Tanzania, Kenya, Uganda, Angola, Mozambique, Ghana, Madagascar, Cameroon, Cote d’Ivoire, Niger, Burkina Faso, Mali, Malawi, Zambia, Senegal, Chad, Somalia, Zimbabwe, Guinea, Rwanda, Benin, Berundi, Tunisia, South Sudan, Togo, Sierra Leone, Congo, Liberia, Central African Republic, Mauritania, Eritrea, Namibia, Gambia, Botswana, Gabon, Lesotho, Guinea-Bissau, Equatorial Guinea, Mauritius, Eswatini.*

**Geographical regions**

*Latin**America, Middle East, Asia, Africa*

Searches were carried out individually for each country and geographical region using the terms listed above.

**Appendix 2 – Questions/topics covered in semi-structured virtual interviews**

1. What are the best sources of epidemiological data for CF in your country?
   1. Are these fully representative?
2. What is your knowledge of the number of CF patients within your country?
3. What is the status of access to CFTR modulators in your country?

**Diagram

Description automatically generated**

**Appendix 3 – Flow diagram depicting provenance of country-level diagnosed CF patient population data**

For 64 countries no data was available.

**Appendix 4 – Country-specific diagnosed patient data**

|  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- |
| Country | CF patient population | Total population (thousands) | Estimated CF prevalence (per 10,000) | Diagnosed source | Reference |
| Europe | | | | | |
| Austria | 816 | 8791 | 0.928 | Registry | 1 |
| Belgium | 1314 | 11560 | 1.137 | Registry | 1 |
| Bulgaria | 193 | 7057 | 0.273 | Registry | 1 |
| Cyprus | 26 | 1267 | 0.205 | Registry | 1 |
| Czechia | 636 | 10683 | 0.595 | Registry | 1 |
| Denmark | 528 | 5809 | 0.909 | Registry | 1 |
| France | 6940 | 67382 | 1.030 | Registry | 1 |
| Germany | 6361 | 80383 | 0.791 | Registry | 1 |
| Greece | 611 | 10669 | 0.573 | Registry | 1 |
| Hungary | 498 | 9810 | 0.508 | Registry | 1 |
| Ireland | 1288 | 5066 | 2.542 | Registry | 1 |
| Italy | 5531 | 62187 | 0.889 | Registry | 1 |
| Latvia | 42 | 1924 | 0.218 | Registry | 1 |
| Lithuania | 28 | 2797 | 0.100 | Registry | 1 |
| Luxembourg | 39 | 606 | 0.644 | Registry | 1 |
| Malta | No data available | 449 | N/A | No data available | No data available |
| Netherlands | 1473 | 17145 | 0.859 | Registry | 1 |
| Poland | 917 | 38412 | 0.239 | Registry | 1 |
| Portugal | 307 | 10342 | 0.297 | Registry | 1 |
| Romania | 224 | 21464 | 0.104 | Registry | 1 |
| Slovakia | 308 | 5444 | 0.566 | Registry | 1 |
| Slovenia | 113 | 2102 | 0.538 | Registry | 1 |
| Spain | 2320 | 47262 | 0.491 | Registry | 1 |
| Sweden | 689 | 10031 | 0.687 | Registry | 1 |
| UK | 10509 | 65074 | 1.615 | Registry | 1 |
| Estonia | 46 | 1244 | 0.370 | Literature | 2 |
| Switzerland | 983 | 8288 | 1.186 | Registry | 1 |
| Norway | 294 | 5371 | 0.547 | Registry | 1 |
| Ukraine | 208 | 43944 | 0.047 | Registry | 1 |
| Russia | 3425 | 143005 | 0.240 | Registry | 1 |
| Albania | 128 | 3064 | 0.418 | Registry | 1 |
| Armenia | 33 | 3038 | 0.109 | Registry | 1 |
| Croatia | 132 | 4272 | 0.309 | Registry | 1 |
| Serbia | 198 | 7075 | 0.280 | Registry | 1 |
| Moldova | 64 | 3433 | 0.186 | Registry | 1 |
| North Macedonia | 127 | 2119 | 0.599 | Registry | 1 |
| Belarus | 111 | 9519 | 0.117 | Registry | 1 |
| Georgia | 72 | 4926 | 0.146 | Registry | 1 |
| Finland | 64 | 5538 | 0.116 | Literature | 3 |
| Country | **CF patient population** | **Total population (thousands)** | **Estimated CF prevalence (per 10,000)** | **Diagnosed source** | **Reference** |
| Bosnia and Herzegovina | 40 | 3847 | 0.104 | Literature | 4 |
| Iceland | 14 | 344 | 0.407 | Registry | 5 |
| North America | | | | | |
| USA | 31199 | 326688 | 0.955 | Registry | 6 |
| Canada | 4344 | 37044 | 1.173 | Registry | 7 |
| Mexico | 950 | 126053 | 0.075 | CF expert | 8 |
| Guatemala | 40 | 16573 | 0.024 | CF expert | 9 |
| Cuba | 296 | 11116 | 0.266 | Literature | 10 |
| Haiti | No data available | N/A | N/A | No data available | No data available |
| Dominican Republic | 61 | 10298 | 0.059 | Literature | 11 |
| Honduras | 2 | 9000 | 0.002 | Literature | 12 |
| Nicaragua | No data available | N/A | N/A | No data available | No data available |
| El Salvador | No data available | N/A | N/A | No data available | No data available |
| Costa Rica | 110 | 4985 | 0.221 | CF expert | 13 |
| Panama | No data available | N/A | N/A | No data available | No data available |
| Jamaica | No data available | N/A | N/A | No data available | No data available |
| Trinidad | No data available | N/A | N/A | No data available | No data available |
| Asia | | | | | |
| India | 600 | 1296692 | 0.005 | Literature | 14 |
| China | 93 | 1384689 | 0.001 | Literature | 15 |
| Indonesia | 6 | 268149 | 0.000 | Literature | 14 |
| Pakistan | 150 | 223912 | 0.007 | Literature | [16](https://onlinelibrary.wiley.com/doi/full/10.1111/j.1365-3156.2009.02253.x) |
| Bangladesh | 95 | 159342 | 0.006 | Literature | 14 |
| Japan | 30 | 125918 | 0.002 | CF expert | 14 |
| Philippines | No data available | N/A | N/A | No data available | No data available |
| Vietnam | No data available | N/A | N/A | No data available | No data available |
| Thailand | 40 | 68855 | 0.006 | CF expert | 14 |
| Myanmar | No data available | N/A | N/A | No data available | No data available |
| South Korea | 80 | 51300 | 0.016 | CF expert | 14 |
| North Korea | No data available | N/A | N/A | No data available | No data available |
| Uzbekistan | No data available | N/A | N/A | No data available | No data available |
| Malaysia | 60 | 32426 | 0.019 | CF expert | 14 |
| Nepal | 1 | 29623 | 0.000 | Literature | 17 |
| Sri Lanka | 10 | 22580 | 0.004 | Literature | 18 |
| Kazakhstan | 144 | 18737 | 0.077 | CF expert | 19 |
| Cambodia | No data available | N/A | N/A | No data available | No data available |
| Azerbaijan | 31 | 10047 | 0.031 | CF expert | 20 |
| Tajikistan | No data available | N/A | N/A | No data available | No data available |
| Laos | No data available | N/A | N/A | No data available | No data available |
| Kyrgyzstan | 12 | 5848 | 0.021 | CF expert | 21 |
| Turkmenistan | No data available | N/A | N/A | No data available | No data available |
| Country | **CF patient population** | **Total population (thousands)** | **Estimated CF prevalence (per 10,000)** | **Diagnosed source** | **Reference** |
| Singapore | 18 | 5696 | 0.032 | Literature | 14 |
| Mongolia | No data available | N/A | N/A | No data available | No data available |
| Israel | 589 | 8407 | 0.701 | Registry | 1 |
| Turkey | 1811 | 81228 | 0.223 | Registry | 1 |
| Oman | 149 | 3485 | 0.428 | CF expert | 24 |
| Qatar | 82 | 2366 | 0.347 | Literature | 25 |
| Bahrain | 109 | 1442 | 0.756 | Literature | 26 |
| Saudi Arabia | 396 | 33119 | 0.120 | Literature | 27 |
| Iran | 300 | 83059 | 0.036 | Literature | 14 |
| Jordan | 202 | 10458 | 0.193 | Literature | 28 |
| Palestine | 270 | 4863 | 0.555 | CF expert | 29 |
| Lebanon | 22 | 6094 | 0.036 | Literature | 30 |
| Syria | 25 | 17739 | 0.014 | Literature | 31 |
| UAE | 23 | 9638 | 0.024 | Literature | 32 |
| Kuwait | No data available | N/A | N/A | No data available | No data available |
| Iraq | No data available | N/A | N/A | No data available | No data available |
| Afghanistan | 1 | 34907 | 0.000 | Literature | 33 |
| Yemen | No data available | N/A | N/A | No data available | No data available |
| Oceania | | | | | |
| Australia | 3151 | 24748 | 1.273 | Registry | 22 |
| New Zealand | 501 | 4780 | 1.048 | Registry | 23 |
| Papua New Guinea | No data available | N/A | N/A | No data available | No data available |
| South America | | | | | |
| Brazil | 6240 | 209130 | 0.298 | Registry | 34 |
| Chile | 734 | 17922 | 0.410 | CF expert | 35 |
| Argentina | 1500 | 44964 | 0.334 | CF expert | 36 |
| Colombia | 972 | 48750 | 0.199 | CF expert | 37 |
| Peru | 36 | 31332 | 0.011 | Literature | 38 |
| Venezuela | 300 | 29626 | 0.101 | Literature | 39 |
| Ecuador | 62 | 16489 | 0.038 | Literature | 40 |
| Bolivia | No data available | N/A | N/A | No data available | No data available |
| Paraguay | No data available | N/A | N/A | No data available | No data available |
| Uruguay | 190 | 3371 | 0.564 | CF expert | 41 |
| Africa | | | | | |
| Egypt | 800 | 99407 | 0.080 | CF expert | 42 |
| South Africa | 500 | 55356 | 0.090 | Registry | [43](https://www-sciencedirect-com.iclibezp1.cc.ic.ac.uk/science/article/pii/S1526054220301081#b0085) |
| Algeria | 180 | 41638 | 0.043 | Literature | 44 |
| Libya | 10 | 6622 | 0.015 | Literature | 45 |
| Sudan | 35 | 43111 | 0.008 | Literature | 46 |
| Morocco | 73 | 34868 | 0.021 | CF expert | 47 |
| Nigeria | No data available | N/A | N/A | No data available | No data available |
| Country | **CF patient population** | **Total population (thousands)** | **Estimated CF prevalence (per 10,000)** | **Diagnosed source** | **Reference** |
| Ethiopia | No data available | N/A | N/A | No data available | No data available |
| DR Congo | No data available | N/A | N/A | No data available | No data available |
| Tanzania | No data available | N/A | N/A | No data available | No data available |
| Kenya | No data available | N/A | N/A | No data available | No data available |
| Uganda | No data available | N/A | N/A | No data available | No data available |
| Angola | No data available | N/A | N/A | No data available | No data available |
| Mozambique | No data available | N/A | N/A | No data available | No data available |
| Ghana | No data available | N/A | N/A | No data available | No data available |
| Madagascar | No data available | N/A | N/A | No data available | No data available |
| Cameroon | No data available | N/A | N/A | No data available | No data available |
| Cote d'Ivoire | No data available | N/A | N/A | No data available | No data available |
| Niger | No data available | N/A | N/A | No data available | No data available |
| Burkina Faso | No data available | N/A | N/A | No data available | No data available |
| Mali | No data available | N/A | N/A | No data available | No data available |
| Malawi | No data available | N/A | N/A | No data available | No data available |
| Zambia | No data available | N/A | N/A | No data available | No data available |
| Senegal | No data available | N/A | N/A | No data available | No data available |
| Chad | No data available | N/A | N/A | No data available | No data available |
| Somalia | No data available | N/A | N/A | No data available | No data available |
| Zimbabwe | 1 | 14030 | 0.001 | Literature | 48 |
| Guinea | No data available | N/A | N/A | No data available | No data available |
| Rwanda | 5 | 12185 | 0.004 | Literature | 49 |
| Benin | No data available | N/A | N/A | No data available | No data available |
| Berundi | No data available | N/A | N/A | No data available | No data available |
| Tunisia | 61 | 11515 | 0.053 | Literature | 50 |
| South Sudan | No data available | N/A | N/A | No data available | No data available |
| Togo | No data available | N/A | N/A | No data available | No data available |
| Sierra Leone | No data available | N/A | N/A | No data available | No data available |
| Congo | No data available | N/A | N/A | No data available | No data available |
| Liberia | No data available | N/A | N/A | No data available | No data available |
| Central African Republic | No data available | N/A | N/A | No data available | No data available |
| Mauritania | No data available | N/A | N/A | No data available | No data available |
| Eritrea | No data available | N/A | N/A | No data available | No data available |
| Namibia | No data available | N/A | N/A | No data available | No data available |
| Gambia | No data available | N/A | N/A | No data available | No data available |
| Botswana | No data available | N/A | N/A | No data available | No data available |
| Gabon | No data available | N/A | N/A | No data available | No data available |
| Lesotho | No data available | N/A | N/A | No data available | No data available |
| Guinea-Bissau | No data available | N/A | N/A | No data available | No data available |
| Equatorial Guinea | No data available | N/A | N/A | No data available | No data available |
| Mauritius | No data available | N/A | N/A | No data available | No data available |
| Eswatini | No data available | N/A | N/A | No data available | No data available |

Population data for each country was obtained from the US Census Bureau international database, for the year 2018 as this was the year most data within the set was obtained.

Prevalence estimates were calculated using estimated diagnosed population data as there were more data available.

Diagram

Description automatically generated

**Appendix 5 – Flow diagram depicting provenance of country-level undiagnosed CF patient population estimates**

For 109 countries no data was available.

**Appendix 6 - Country-specific total patient data (including undiagnosed)**

|  |  |  |  |  |  |  |  |  |
| --- | --- | --- | --- | --- | --- | --- | --- | --- |
| Country | Estimated total CF population low interval | Estimated total CF population (inc. undiagnosed) | Estimated total CF population high interval | Coverage figure used | Implied total estimated CF prevalence (per 10,000) | Implied estimated percentage of total cases diagnosed | Estimate source | Reference |
| Europe | | | | | | | | |
| Austria | 816 | 859 | 907 | 95% | 0.977 | 95% | Registry | 1 |
| Belgium | 1314 | 1383 | 1460 | 95% | 1.197 | 95% | Registry | 1 |
| Bulgaria | 193 | 227 | 276 | 85% | 0.322 | 85% | Registry | 1 |
| Cyprus | 26 | 29 | 33 | 90% | 0.228 | 90% | Registry | 1 |
| Czechia | N/A | 642 | N/A | 99% | 0.601 | 99% | Registry | 1 |
| Denmark | 533 | 533 | 533 | 99% | 0.918 | 99% | Registry | 1 |
| France | 6940 | 7305 | 7711 | 95% | 1.084 | 95% | Registry | 1 |
| Germany | N/A | 7068 | N/A | 90% | 0.879 | 90% | Registry | 1 |
| Greece | 611 | 627 | 643 | 97·50% | 0.587 | 97·50% | Registry | 1 |
| Hungary | N/A | 524 | N/A | 95% | 0.534 | 95% | Registry | 1 |
| Ireland | N/A | 1356 | N/A | 95% | 2.676 | 95% | Registry | 1 |
| Italy | N/A | 5673 | N/A | 97·50% | 0.912 | 97·50% | Registry | 1 |
| Latvia | 42 | 44 | 47 | 95% | 0.230 | 95% | Registry | 1 |
| Lithuania | N/A | 54 | N/A | 52% | 0.193 | 52% | Registry | 1 |
| Luxembourg | 39 | 40 | 41 | 97·50% | 0.660 | 98% | Registry | 1 |
| Malta | N/A | 26 | N/A | No data available | 0.579 | N/A | Literature | 3 |
| Netherlands | 1473 | 1551 | 1637 | 95% | 0.904 | 95% | Registry | 1 |
| Poland | 917 | 1146 | 1528 | 80% | 0.298 | 80% | Registry | 1 |
| Portugal | 307 | 315 | 323 | 97·50% | 0.304 | 97·50% | Registry | 1 |
| Romania | N/A | 640 | N/A | 35% | 0.298 | 35% | Registry | 1 |
| Slovakia | 308 | 324 | 342 | 95% | 0.596 | 95% | Registry | 1 |
| Slovenia | 113 | 116 | 119 | 97·50% | 0.551 | 97·50% | Registry | 1 |
| Spain | N/A | 3093 | N/A | 75% | 0.655 | 75% | Registry | 1 |
| Sweden | 689 | 707 | 725 | 97·50% | 0.704 | 97·50% | Registry | 1 |
| UK | N/A | 10615 | N/A | 99% | 1.631 | 99% | Registry | 1 |
| Estonia | N/A | 83 | N/A | No data available | 0.667 | 55% | Literature | 51 |
| Switzerland | 983 | 1008 | 1035 | 97·50% | 1.216 | 97·50% | Registry | 1 |
| Norway | 294 | 327 | 368 | 90% | 0.608 | 90% | Registry | 1 |
| Ukraine | 208 | 347 | 1040 | 60% | 0.079 | 60% | Registry | 1 |
| Russia | 3425 | 3513 | 3605 | 98% | 0.246 | 98% | Registry | 1 |
| Albania | N/A | 160 | N/A | 90% | 0.464 | 90% | Registry | 1 |
| Armenia | 33 | 39 | 47 | 85% | 0.128 | 85% | Registry | 1 |
| Croatia | 132 | 135 | 139 | 98% | 0.317 | 98% | Registry | 1 |
| Serbia | 198 | 208 | 220 | 95% | 0.295 | 95% | Registry | 1 |
| Moldova | 64 | 67 | 71 | 95% | 0.196 | 95% | Registry | 1 |
| North Macedonia | 127 | 134 | 141 | 95% | 0.631 | 95% | Registry | 1 |
| Belarus | 111 | 139 | 185 | 80% | 0.146 | 80% | Registry | 1 |
| Country | **Estimated total CF population low interval** | **Estimated total CF population (inc. undiagnosed)** | **Estimated total CF population high interval** | **Coverage figure used** | **Implied total estimated CF prevalence (per 10,000)** | **Implied estimated percentage of total cases diagnosed** | **Estimate source** | **Reference** |
| Georgia | 72 | 80 | 90 | 90% | 0.162 | 90% | Registry | 1 |
| Asia | | | | | | | | |
| India | 21429 | 37406 | 58970 | No data available | 0.288 | 2% | Literature | 52 |
| Japan | N/A | 360 | N/A | No data available | 0.029 | 8% | Literature | 53 |
| Israel | 589 | 604 | 620 | 97·50% | 0.719 | 97·50% | Registry | 1 |
| Turkey | 3150 | 3500 | 3850 | No data available | 0.431 | 52% | CF Expert | 54 |
| Oman | N/A | 602 | N/A | No data available | 1.727 | 25% | Literature | 55 |
| UAE | N/A | 617 | N/A | No data available | 0.640 | 4% | Literature | 32 |
| Oceania | | | | | | | | |
| New Zealand | N/A | 511 | N/A | 98% | 1.070 | 98% | Registry | 23 |
| South America | | | | | | | | |
| Argentina | 2300 | 2500 | 2700 | No data available | 0.556 | 60% | CF Expert | 36 |
| Uruguay | N/A | 234 | N/A | No data available | 0.694 | 81% | Literature | 56 |
| Africa | | | | | | | | |
| Egypt | N/A | 5000 | N/A | No data available | 0.503 | 16% | CF Expert | 42 |
| Morocco | N/A | 8495 | N/A | No data available | 2.436 | 1% | Literature | 57 |

**Appendix 7 – Flowchart depicting the process used to generate the estimate of the undiagnosed CF population in India**

*Diagram

Description automatically generated*

Prevalence estimates were found derived from the study of Indian migrant populations in the USA and Canada.58,59 A mean of the expected CF populations following from these values was calculated alongside standard deviation to generate our final estimate.60

NB For *Mei-Zahav et al.* prevalence estimates are presented per specific age group, excluding those aged over 44. For the purposes of our analysis these samples were combined so that prevalence was calculated using the total listed population and identified CF cases.

**Appendix 8 – Exact figures used in calculation of Trikafta access61**

|  |  |  |  |  |
| --- | --- | --- | --- | --- |
| Region | Revenue proportion | | | |
| **2020** | **2019** | **2018** | **3 year average** |
| US | 78% | 74% | 78% | 76% |
| EU | 18% | 21% | 18% | 19% |
| Other ex. US | 4% | 5% | 5% | 5% |
|  | | | | |
| Total Trikafta Q4 2020 revenues | 1091000000 | | | |
|  | | | | |
| Region | **Geographically weighted Trikafta revenue (USD)** | | **Lowest 3-month price available (USD)** | |
| US | 883710000 | | 58322 | |
| EU | 208210760 | | 47713 | |
|  | | | | |
| Region | **Estimated number of patients treated with Trikafta** | | | |
| US | 15152 | | | |
| EU | 4364 | | | |
| Total | 19516 | | | |

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