

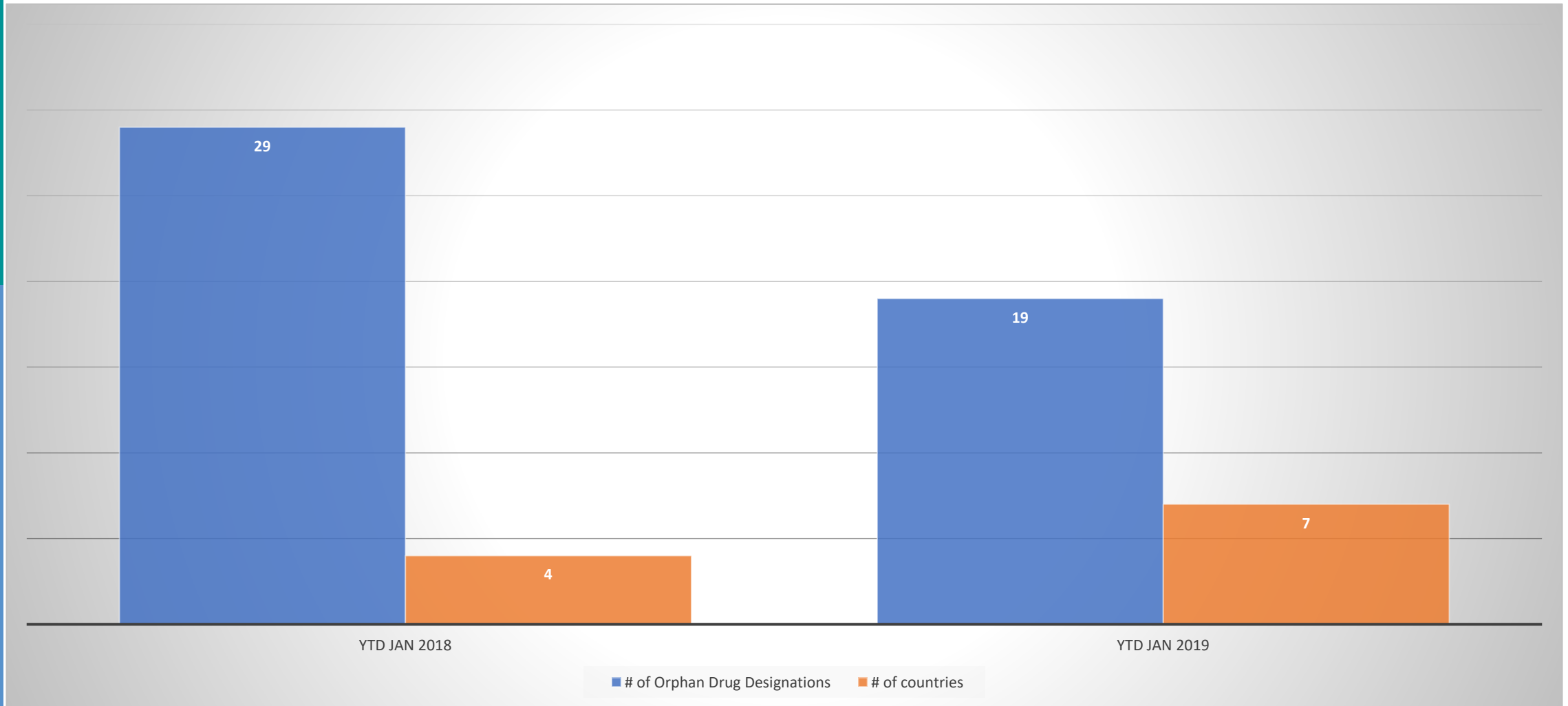
Orphan Drug Designations in the USA 2019

K&A Schaefer Consulting

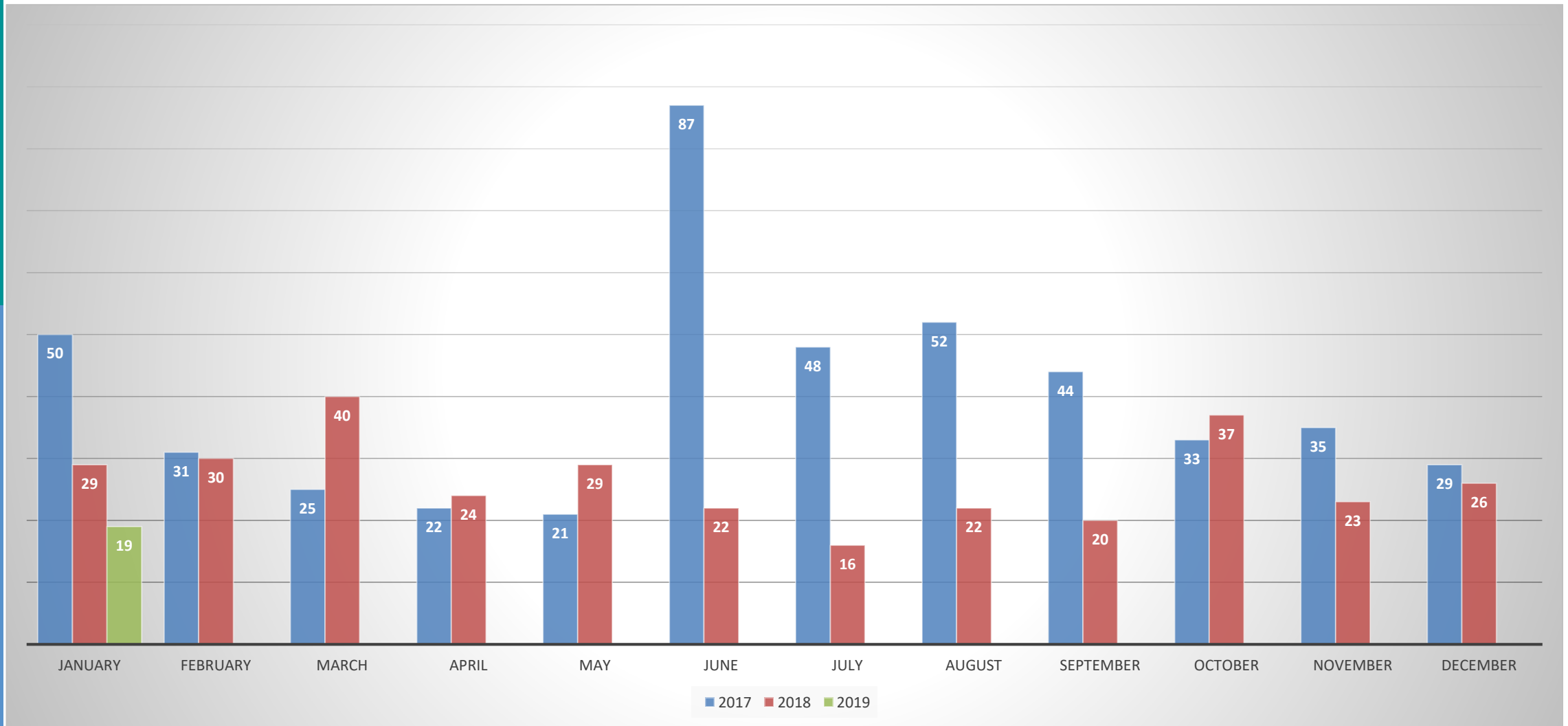
Summary January 2019

- January 2019 was impacted by the partial government shutdown and only 19 Orphan Drug Designations have been approved
 - In January 2018 a total of 29 have been approved
 - 34% less designations have been approved compared to last year which was already 42% lower than 2017.
- Companies from a total of 7 countries received at least one Orphan Drug Designation from the FDA in 2018
 - 63% of all Orphan Drug Designations come from the USA
 - In January 2018 Orphan Drug Designations from 4 countries were approved by FDA
- In January 2019 a total of 17 companies received at least one Orphan Drug designation. 16 received one designation which is 94% of all designations
 - 1 company received three designations

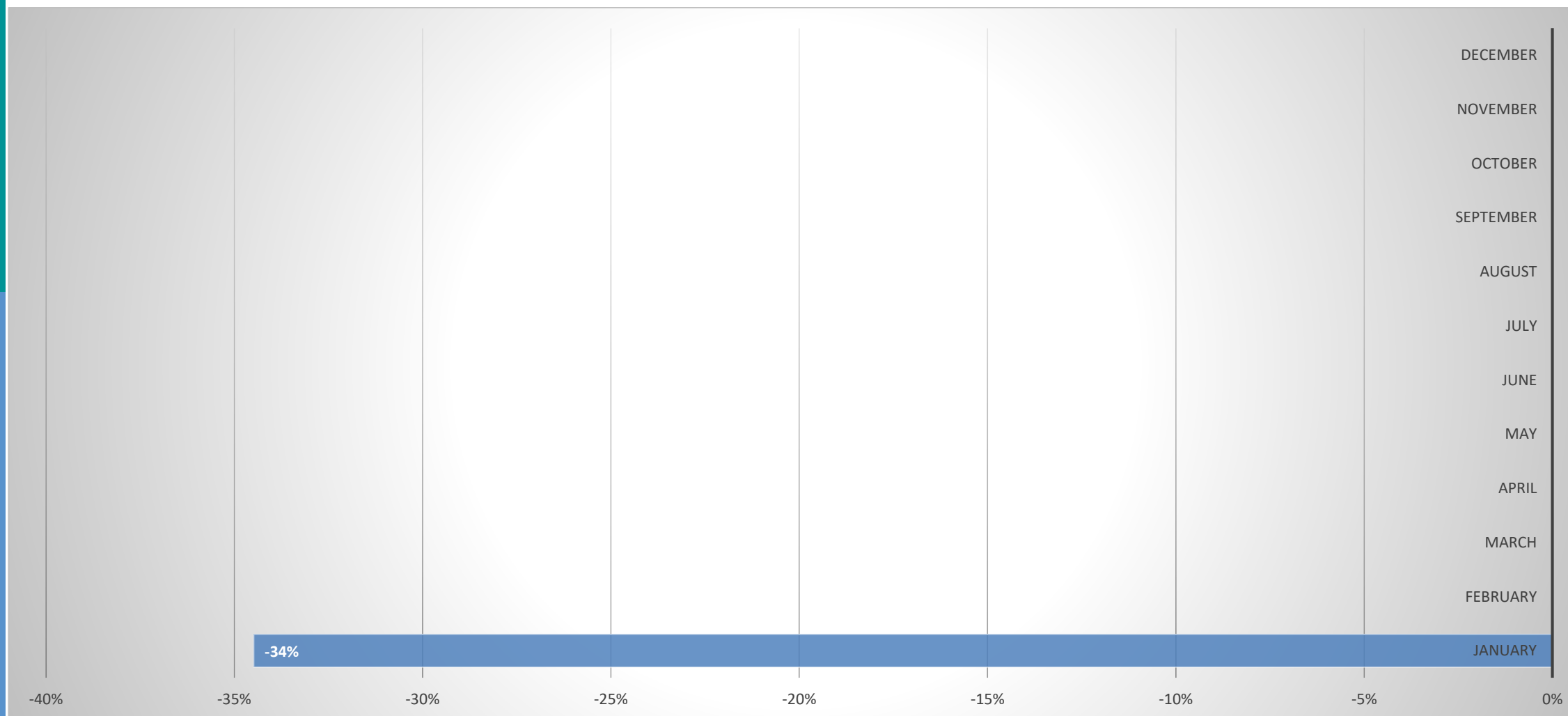
The Total Number Of Orphan Drug Designations declined by 34% YTD Jan due to the partial shutdown



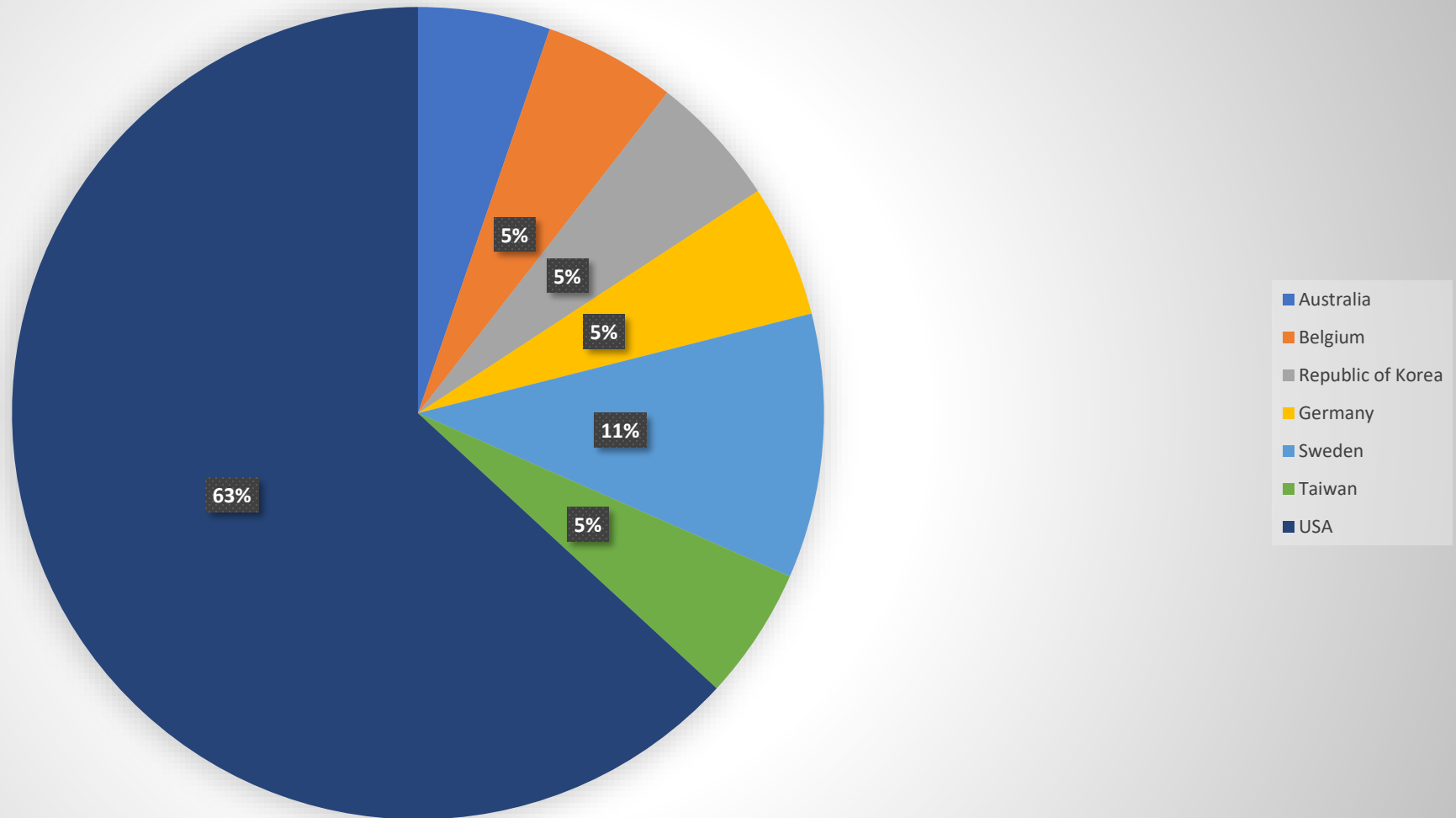
Designations 2017 vs 2018 vs 2019 on a monthly basis



Development 2018 vs 2019 on a monthly basis in %



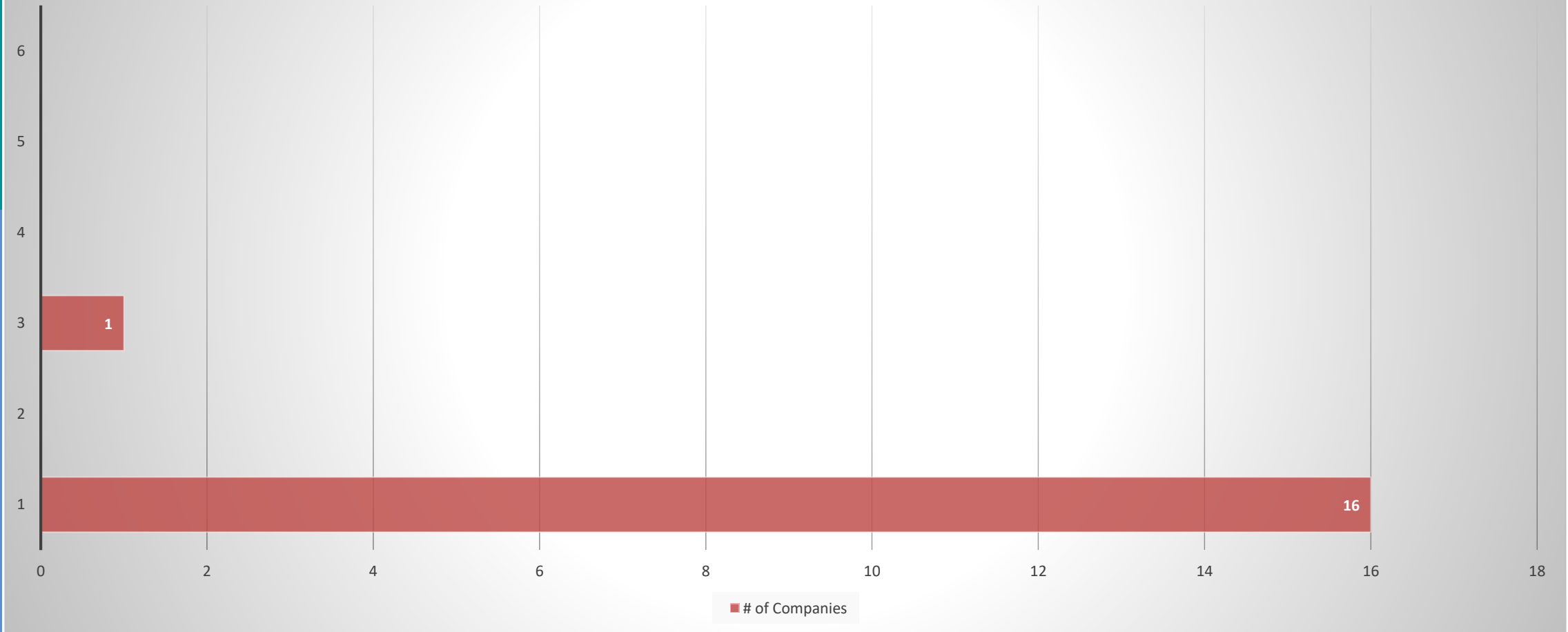
The Vast Majority Of Orphan Drug Designations Are From The USA



94% Of All Companies Received Approval for One Orphan Drug Designation for YTD Jan 2019



Number of designations per company



Development of Orphan Drug Designations over time

Development of Orphan Drug Designation Approvals over time

