

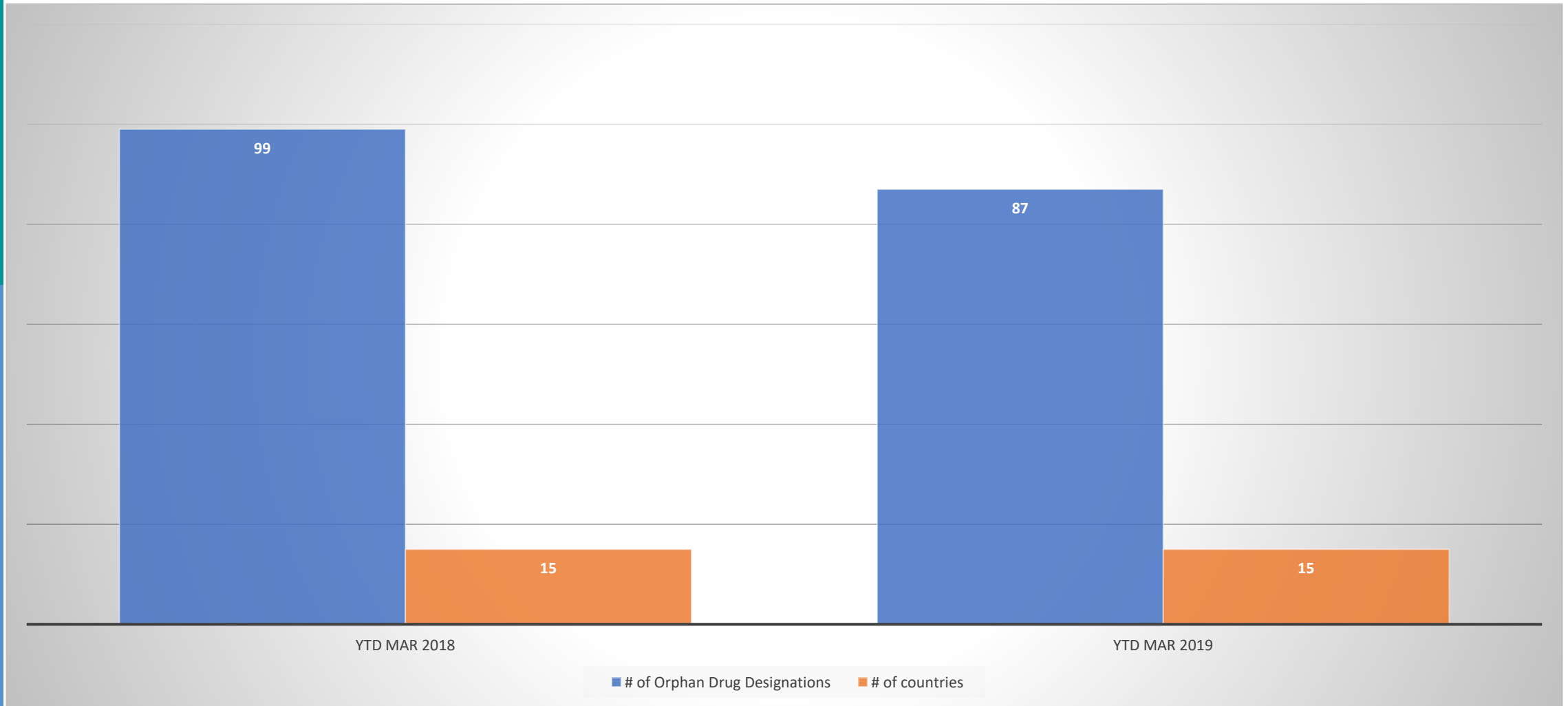
# Orphan Drug Designations in the USA 2019

K&A Schaefer Consulting

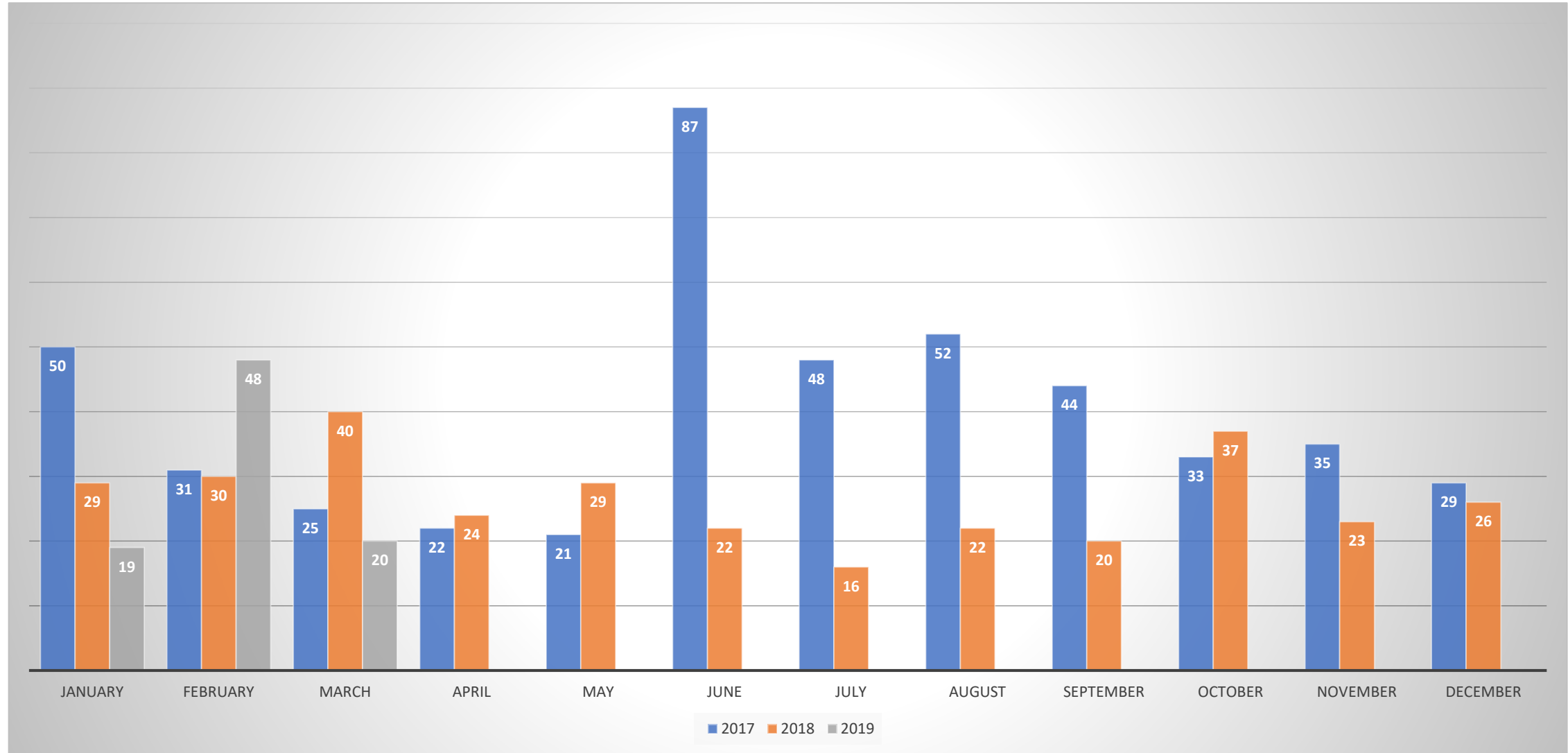
## Summary March 2019

- March 2019 – a total of 20 Orphan Drug designations have been approved, a 58% reduction compared with February
  - In March 2018 a total of 40 have been approved
  - 50% less designations have been approved compared to last year
- YTD March 2019 a total of 87 Orphan Drug designations have been approved, 12% less than for the same period in 2018.
  - YTD March 2019 is 18% lower than the same period in 2017.
- Companies from a total of 15 countries received at least one Orphan Drug Designation from the FDA YTD March 2019
  - 70% of all Orphan Drug Designations come from the USA
  - YTD March 2018 Orphan Drug Designations from 15 countries were approved by FDA
- YTD March 2019 a total of 72 companies received at least one Orphan Drug designation. 61 received one designation which is 86% of all designations
  - 1 company received four designations so far.

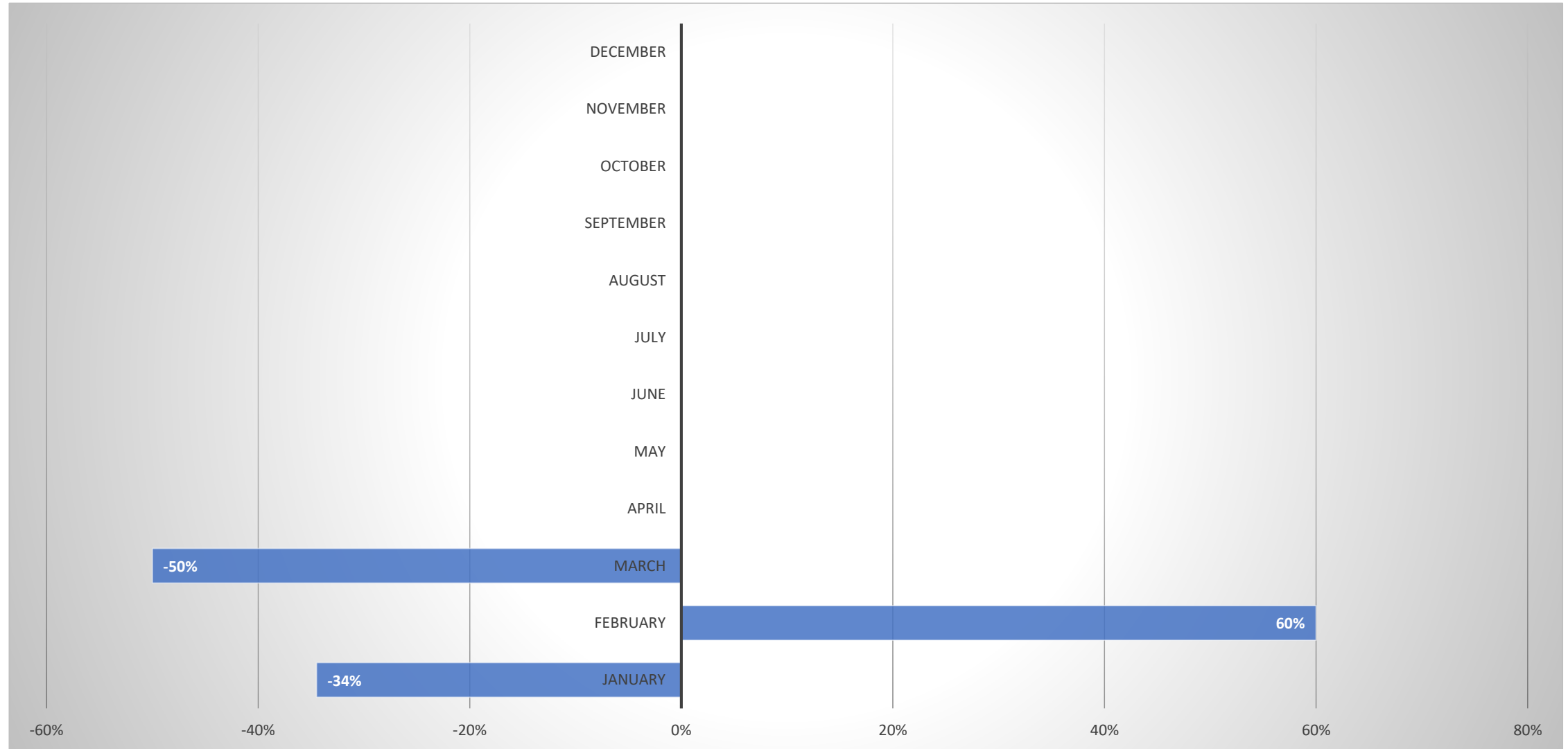
# The Total Number Of Orphan Drug Designations decreased by 18% YTD March



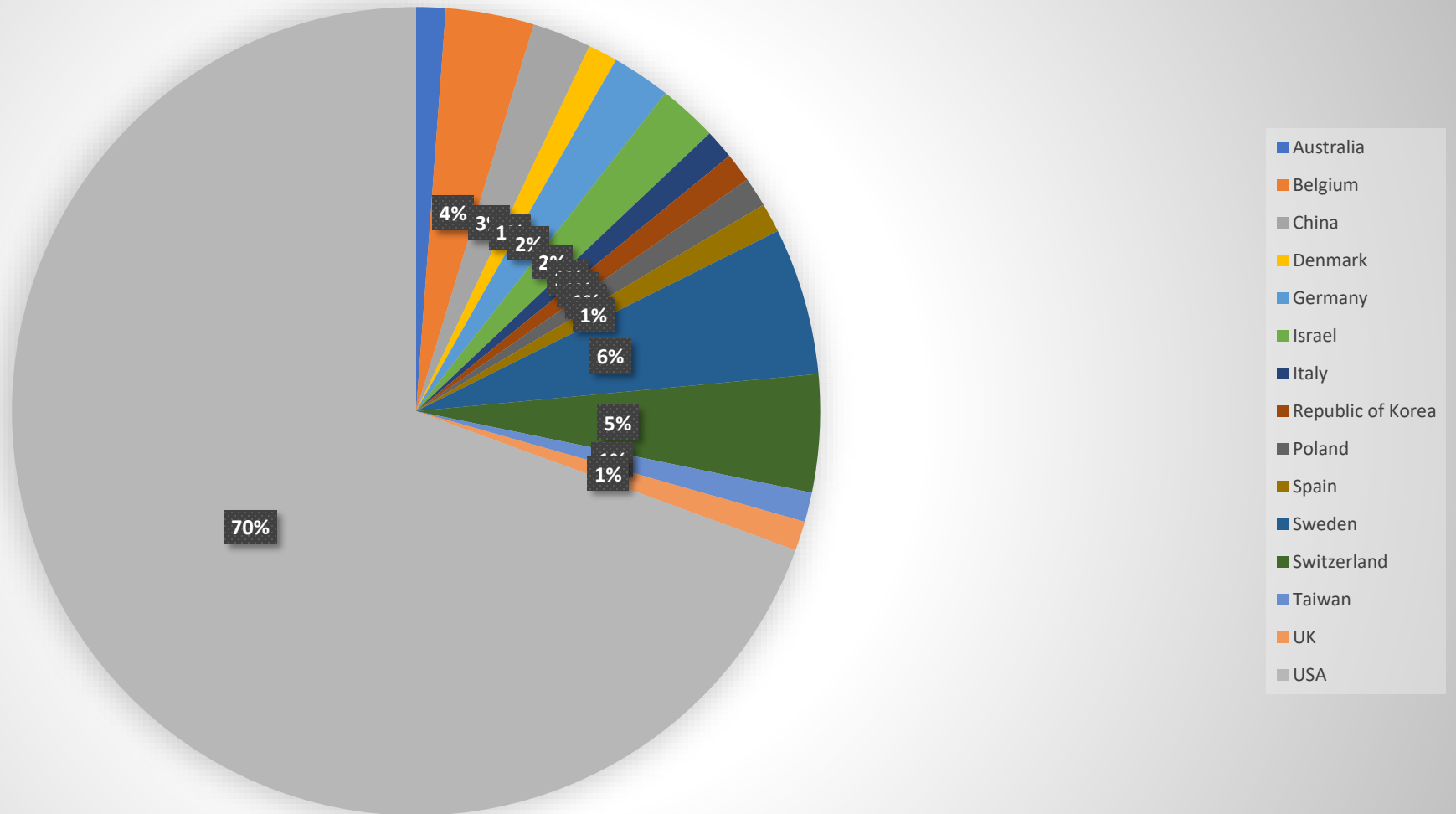
# Designations 2017 vs 2018 vs 2019 on a monthly basis



## Development 2018 vs 2019 on a monthly basis in %



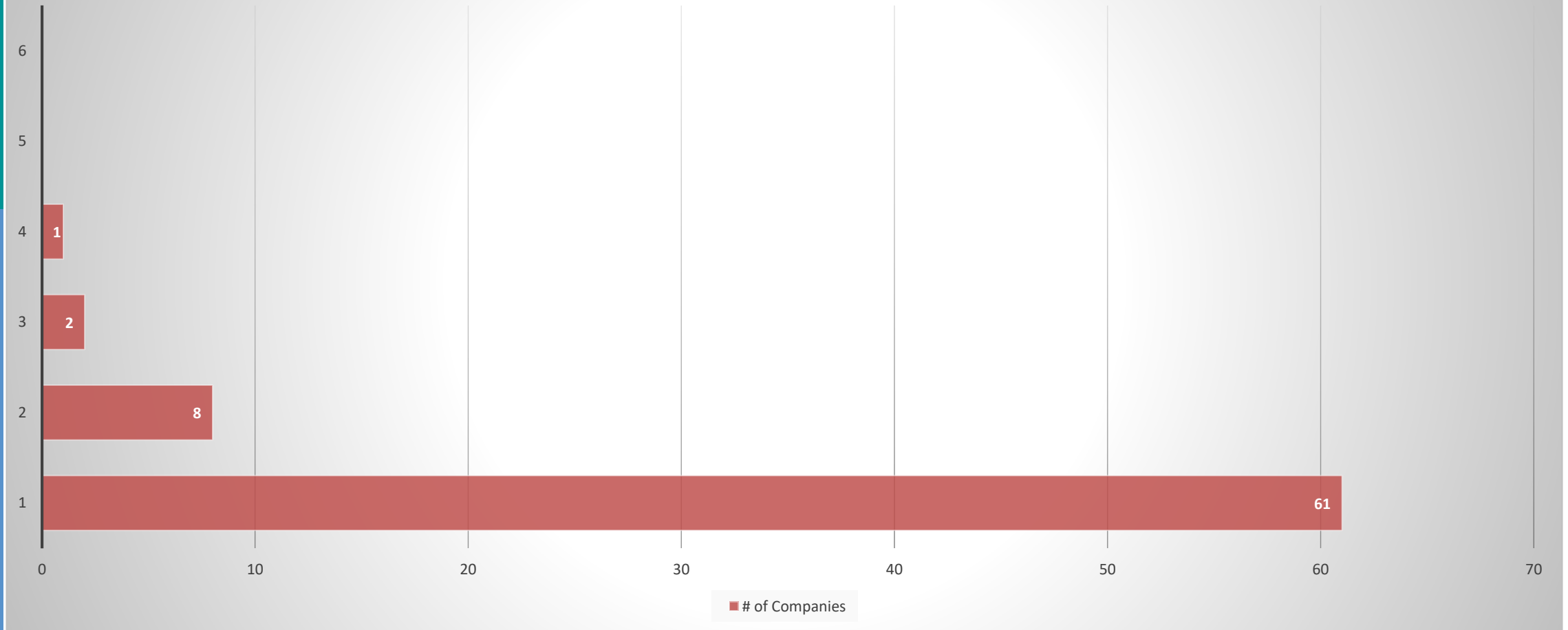
# The Vast Majority Of Orphan Drug Designations Are From The USA



# 85% (-1%) Of All Companies Received Approval for One Orphan Drug Designation for YTD Mar 2019



Number of designations per company



# Development of Orphan Drug Designations over time

