Table 1: Summary statistics for selected variables

| Table 1. Building statistics for science var | | ll trials | J | JS trials |
|--|---------|--------------|--------|------------|
| | Mean | Observations | Mean | Observatio |
| Uses biomarker | 0.4092 | 131,971 | 0.4619 | 49,540 |
| Generous PPM | 0.0643 | 131,971 | 0.0907 | 49,540 |
| Restrictive PPM | 0.0581 | 131,971 | 0.0813 | $49,\!540$ |
| Biomarker role: disease | 0.0842 | 131,971 | 0.1145 | $49,\!540$ |
| Biomarker role: toxic effect | 0.0496 | 131,971 | 0.0699 | $49,\!540$ |
| Biomarker role: therapeutic effect | 0.3371 | 131,971 | 0.3758 | $49,\!540$ |
| Biomarker role: not determined | 0.0023 | 131,971 | 0.0024 | $49,\!540$ |
| Biomarker type: anthropomorphic | 0.0350 | 131,971 | 0.0400 | $49,\!540$ |
| Biomarker type: biochemical | 0.1248 | 131,971 | 0.1300 | $49,\!540$ |
| Biomarker type: cellular | 0.0308 | 131,971 | 0.0424 | $49,\!540$ |
| Biomarker type: genomic | 0.2321 | 131,971 | 0.2845 | 49,540 |
| Biomarker type: physiological | 0.0849 | 131,971 | 0.0865 | $49,\!540$ |
| Biomarker type: proteomic | 0.2426 | 131,971 | 0.2942 | $49,\!540$ |
| Biomarker type: structural (imaging) | 0.0177 | 131,971 | 0.0200 | $49,\!540$ |
| Biomarker role (detailed): diagnosis | 0.2948 | 117,180 | 0.3448 | 43,777 |
| Biomarker role (detailed): differential diagnosis | 0.1829 | 117,180 | 0.2041 | 43,777 |
| Biomarker role (detailed): predicting drug resistance | 0.0624 | 117,180 | 0.0778 | 43,777 |
| Biomarker role (detailed): predicting treatment efficacy | 0.2568 | 117,180 | 0.3060 | 43,777 |
| Biomarker role (detailed): predicting treatment toxicity | 0.0474 | 117,180 | 0.0493 | 43,777 |
| Biomarker role (detailed): screening | 0.0523 | 117,180 | 0.0547 | 43,777 |
| Biomarker role (detailed): selection for therapy | 0.0938 | 117,180 | 0.1111 | 43,777 |
| Phase 1 Clinical | 0.3305 | 131,971 | 0.3653 | $49,\!540$ |
| Phase 2 Clinical | 0.4367 | 131,971 | 0.4263 | $49,\!540$ |
| Phase 3 Clinical | 0.2328 | 131,971 | 0.2083 | $49,\!540$ |
| Drug indication for neoplasm | 0.3340 | 131,971 | 0.3990 | $49,\!540$ |
| Received NIH funding | 0.0282 | 131,971 | 0.0703 | $49,\!540$ |
| Trial site in US | 0.4368 | 113,410 | 1.0000 | $49,\!540$ |
| N | 131,971 | | 49,540 | |

 $^{../ {\}it figures/01a-trial}_c ount_b y_p hase. eps../figures/01b-trial_g rowth_b y_p hase. eps../figures/02a-bmkr_configures/01a-trial_g rowth_b y_p hase. eps../figures/01a-trial_g rowth_b y_p hase. eps../figures/01a-t$

Table 2: Number of trials employing biomarkers by type

| Table 2. Number of thats employing blomarkers by type | | | | | | | |
|---|---------------|-----------------|-------------|----------|-----------|---------------|---------------------------------|
| | Any biomarker | Anthropomorphic | Biochemical | Cellular | Genomic | Physiological | Pro |
| Overall | 53,998 | 4,620 | 16,472 | 4,070 | 30,634 | 11,205 | 32 |
| 1995 | 105 | 4 | 29 | 1 | 59 | 22 | |
| 1996 | 131 | 5 | 34 | 6 | 77 | 16 | |
| 1997 | 193 | 10 | 62 | 8 | 119 | 24 | |
| 1998 | 288 | 12 | 74 | 6 | 165 | 58 | |
| 1999 | 448 | 16 | 119 | 22 | 292 | 68 | ; |
| 2000 | 542 | 33 | 149 | 28 | 349 | 83 | ; |
| 2001 | 645 | 36 | 190 | 38 | 406 | 94 | 4 |
| 2002 | 869 | 53 | 263 | 36 | 558 | 135 | ļ |
| 2003 | 1,085 | 80 | 358 | 51 | 698 | 156 | , |
| 2004 | 1,524 | 126 | 469 | 68 | 950 | 216 | 9 |
| 2005 | 1,928 | 135 | 580 | 118 | $1,\!157$ | 314 | 1 |
| 2006 | 2,280 | 178 | 737 | 138 | 1,379 | 377 | 1 |
| 2007 | 2,718 | 220 | 831 | 207 | 1,687 | 437 | 1 |
| 2008 | 3,005 | 252 | 970 | 245 | 1,813 | 548 | 1 |
| 2009 | 3,492 | 288 | 1,137 | 251 | 2,157 | 627 | 2 |
| 2010 | 3,916 | 334 | 1,239 | 304 | 2,333 | 740 | 2 |
| 2011 | 4,228 | 366 | 1,353 | 357 | $2,\!525$ | 828 | 2 |
| 2012 | 4,517 | 408 | 1,463 | 406 | 2,566 | 994 | 2 |
| 2013 | 4,681 | 439 | 1,446 | 382 | $2,\!544$ | 1,104 | 2 |
| 2014 | 5,099 | 518 | 1,576 | 434 | 2,647 | 1,310 | 2 2 2 2 2 2 3 |
| 2015 | 5,857 | 546 | 1,610 | 438 | 2,944 | 1,499 | 3 |
| 2016 | 6,447 | 561 | 1,783 | 526 | 3,209 | 1,555 | 3 |

Table 3: Number of trials employing biomarkers by detailed role

| 1a | Table 3: Number of trials employing blomarkers by detailed role | | | | | | | |
|---------|---|------------|--------------|-----------------------|------------|------------|-----------|----------|
| | | | | Predicting | Predicting | Predicting | | Sele |
| | Any | | Differential | drug | treatment | treatment | | fe |
| | biomarker | Diagnosis | Diagnosis | resistance | efficacy | toxicity | Screening | the |
| Overall | 39,207 | $34,\!545$ | 21,429 | 7,312 | 30,091 | $5,\!556$ | $6{,}133$ | 10, |
| 1995 | 105 | 68 | 45 | 7 | 62 | 8 | 8 | 1 |
| 1996 | 131 | 88 | 49 | 22 | 81 | 14 | 14 | 1 |
| 1997 | 193 | 130 | 83 | 38 | 122 | 31 | 39 | 3 |
| 1998 | 288 | 210 | 137 | 66 | 199 | 53 | 42 | 6 |
| 1999 | 448 | 341 | 201 | 76 | 310 | 85 | 57 | S |
| 2000 | 542 | 369 | 233 | 88 | 343 | 78 | 59 | 1 |
| 2001 | 645 | 458 | 275 | 121 | 421 | 85 | 81 | 13 |
| 2002 | 869 | 624 | 395 | 151 | 578 | 122 | 109 | 20 |
| 2003 | 1,085 | 764 | 487 | 174 | 691 | 157 | 132 | 20 33 |
| 2004 | 1,524 | 1,051 | 675 | 240 | 954 | 224 | 190 | 3 |
| 2005 | 1,928 | 1,306 | 799 | 286 | 1,189 | 263 | 239 | 4 |
| 2006 | 2,280 | 1,575 | 1,004 | 370 | 1,396 | 308 | 291 | 5 |
| 2007 | 2,718 | 1,882 | 1,215 | 444 | 1,693 | 369 | 332 | 6 |
| 2008 | 3,005 | 2,046 | 1,360 | 496 | 1,832 | 430 | 362 | 6 |
| 2009 | 3,492 | $2,\!352$ | 1,578 | 649 | 2,145 | 504 | 482 | 8 |
| 2010 | 3,916 | 2,539 | 1,540 | 581 | 2,210 | 343 | 444 | 7 |
| 2011 | 4,228 | 2,738 | 1,698 | 582 | 2,379 | 376 | 502 | 8 |
| 2012 | 4,517 | 2,909 | 1,780 | 574 | 2,494 | 376 | 462 | 90 |
| 2013 | 4,681 | 2,932 | 1,778 | 609 | 2,530 | 396 | 500 | 9 |
| 2014 | 5,099 | 3,071 | 1,809 | 548 | $2,\!552$ | 409 | 519 | 93 |
| 2015 | 5,857 | 3,355 | 2,005 | 574 | 2,816 | 427 | 589 | 1,0 |
| 2016 | 6,447 | 3,737 | 2,283 | 616 | 3,094 | 498 | 680 | 1,1 |
| | | | | | | | | |

Table 4: Potential precision medicine trials (1995-2016): Generous precision medicine definition

| medicii | ne definition | | | | | |
|---------|---------------|---------------------|-------------|--------------------|-------------|-------------|
| | (1) | (2) | (3) | (4) | (5) | (6) |
| | PPM Trials: | | PPM Trials: | Share of | PPM Trials: | Share |
| | All | Share of trials (%) | Phase I | Phase I trials (%) | Phase II | Phase II to |
| 1995 | 12 | 1.39 | 4 | 1.2 | 2 | .63 |
| 1996 | 25 | 2.58 | 6 | 1.77 | 10 | 2.5 |
| 1997 | 37 | 2.8 | 11 | 2.44 | 16 | 3.1 |
| 1998 | 56 | 3.29 | 10 | 1.72 | 31 | 4.4 |
| 1999 | 75 | 3.12 | 26 | 3.03 | 37 | 3.9 |
| 2000 | 95 | 3.62 | 27 | 3.03 | 48 | 4.5 |
| 2001 | 114 | 3.81 | 41 | 4.13 | 50 | 4.0 |
| 2002 | 144 | 3.87 | 46 | 3.99 | 70 | 4.4 |
| 2003 | 166 | 3.96 | 45 | 3.55 | 85 | 4.8 |
| 2004 | 234 | 4.49 | 68 | 4.48 | 126 | 5.6 |
| 2005 | 263 | 4.1 | 67 | 3.63 | 143 | 5.0 |
| 2006 | 299 | 4.17 | 74 | 3.44 | 167 | 5.4 |
| 2007 | 407 | 5.39 | 109 | 4.62 | 231 | 6.9 |
| 2008 | 408 | 5.09 | 116 | 4.34 | 229 | 6.6 |
| 2009 | 563 | 6.63 | 178 | 5.95 | 300 | 8.2 |
| 2010 | 563 | 6.44 | 185 | 5.97 | 311 | 8.5 |
| 2011 | 642 | 7.14 | 214 | 6.88 | 361 | 9.3 |
| 2012 | 699 | 7.54 | 231 | 7.6 | 381 | 9.1 |
| 2013 | 781 | 8.55 | 257 | 8.44 | 396 | 9.8 |
| 2014 | 836 | 8.85 | 337 | 9.95 | 388 | 9.4 |
| 2015 | 1,009 | 9.55 | 368 | 10.5 | 482 | 9.8 |
| 2016 | 1,057 | 8.69 | 417 | 10.3 | 501 | 8.4 |

Table 5: Potential precision medicine trials (1995-2016): Restrictive precision

| medicir | ne definition | | (1000 2 010): | Teodoricorro procidion | | |
|---------|---------------|------------------------|----------------------|------------------------|-------------|-------------|
| | (1) | (2) | (3) | (4) | (5) | (6) |
| | PPM Trials: | | PPM Trials: | Share of | PPM Trials: | Share |
| | All | Share of trials $(\%)$ | Phase I | Phase I trials (%) | Phase II | Phase II to |
| 1995 | 9 | 1.04 | 3 | .898 | 2 | .63 |
| 1996 | 23 | 2.37 | 5 | 1.47 | 9 | 2.2 |
| 1997 | 34 | 2.57 | 9 | 2 | 15 | 2.9 |
| 1998 | 53 | 3.11 | 9 | 1.55 | 30 | 4.2 |
| 1999 | 70 | 2.91 | 23 | 2.68 | 35 | 3.7 |
| 2000 | 90 | 3.43 | 25 | 2.8 | 46 | 4.3 |
| 2001 | 105 | 3.51 | 36 | 3.63 | 46 | 3.7 |
| 2002 | 133 | 3.58 | 41 | 3.56 | 66 | 4.2 |
| 2003 | 152 | 3.63 | 37 | 2.92 | 80 | 4.5 |
| 2004 | 212 | 4.06 | 60 | 3.95 | 112 | 5.0 |
| 2005 | 240 | 3.75 | 58 | 3.14 | 131 | 4.6 |
| 2006 | 275 | 3.83 | 64 | 2.98 | 156 | 5.0 |
| 2007 | 370 | 4.9 | 89 | 3.78 | 218 | 6.5 |
| 2008 | 380 | 4.74 | 104 | 3.89 | 217 | 6.3 |
| 2009 | 502 | 5.91 | 148 | 4.95 | 274 | 7.5 |
| 2010 | 514 | 5.88 | 165 | 5.33 | 285 | 7.8 |
| 2011 | 592 | 6.58 | 188 | 6.04 | 343 | 8.8 |
| 2012 | 645 | 6.96 | 209 | 6.88 | 355 | 8.5 |
| 2013 | 720 | 7.88 | 231 | 7.59 | 369 | 9.1 |
| 2014 | 748 | 7.92 | 306 | 9.03 | 343 | 8.3 |
| 2015 | 883 | 8.35 | 322 | 9.21 | 417 | 8.5 |
| 2016 | 914 | 7.52 | 346 | 8.56 | 442 | 7.4 |

Table 6: Potential precision medicine trials (1995-2016): Generous precision medicine definition for drugs with cancer indications

| | (1) | (2) | (3) | (4) | (5) | (6) |
|------|-------------|---------------------|-------------|--------------------|-------------|-------------|
| | PPM Trials: | | PPM Trials: | Share of | PPM Trials: | Share |
| | All | Share of trials (%) | Phase I | Phase I trials (%) | Phase II | Phase II to |
| 1995 | 8 | 2.33 | 3 | 2.38 | 2 | 1.4 |
| 1996 | 24 | 5.3 | 6 | 3.57 | 10 | 5.2 |
| 1997 | 34 | 5.81 | 11 | 4.89 | 13 | 5.1 |
| 1998 | 54 | 6.26 | 10 | 3.15 | 30 | 7.5 |
| 1999 | 67 | 6.41 | 21 | 5.34 | 35 | 7.2 |
| 2000 | 86 | 6.62 | 24 | 4.75 | 45 | 7.5 |
| 2001 | 104 | 7.74 | 39 | 7.39 | 45 | 7.2 |
| 2002 | 137 | 8.78 | 44 | 7.76 | 68 | 8.6 |
| 2003 | 142 | 8.53 | 34 | 5.72 | 80 | 9.4 |
| 2004 | 204 | 10.2 | 61 | 8.93 | 113 | 10. |
| 2005 | 226 | 9.89 | 55 | 6.67 | 130 | 10. |
| 2006 | 261 | 10.7 | 64 | 7.62 | 154 | 11. |
| 2007 | 363 | 14.2 | 87 | 9.61 | 217 | 16. |
| 2008 | 368 | 14.2 | 98 | 10.2 | 221 | 16. |
| 2009 | 504 | 17.9 | 162 | 14.4 | 272 | 19. |
| 2010 | 509 | 18 | 164 | 14.6 | 289 | 20. |
| 2011 | 572 | 19.8 | 189 | 16.7 | 332 | 22. |
| 2012 | 620 | 21.5 | 211 | 18.9 | 341 | 23. |
| 2013 | 678 | 24.7 | 225 | 21.5 | 356 | 25. |
| 2014 | 713 | 26.5 | 296 | 25.6 | 332 | 26. |
| 2015 | 854 | 29.6 | 319 | 27 | 410 | 30. |
| 2016 | 899 | 27.1 | 375 | 27 | 410 | 26. |
| | | | | | | |

Table 7: Potential precision medicine trials (1995-2016): Restrictive precision medicine definition for drugs with cancer indications

| | | 1 41480 11111 0411001 111 | | | | |
|------|-------------|---------------------------|-------------|--------------------|-------------|-------------|
| | (1) | (2) | (3) | (4) | (5) | (6) |
| | PPM Trials: | | PPM Trials: | Share of | PPM Trials: | Share |
| | All | Share of trials (%) | Phase I | Phase I trials (%) | Phase II | Phase II to |
| 1995 | 8 | 2.33 | 3 | 2.38 | 2 | 1.4 |
| 1996 | 22 | 4.86 | 5 | 2.98 | 9 | 4.6 |
| 1997 | 31 | 5.3 | 9 | 4 | 12 | 4.7 |
| 1998 | 52 | 6.03 | 9 | 2.84 | 29 | 7.2 |
| 1999 | 64 | 6.12 | 20 | 5.09 | 33 | 6.8 |
| 2000 | 83 | 6.39 | 22 | 4.36 | 44 | 7.4 |
| 2001 | 100 | 7.45 | 35 | 6.63 | 45 | 7.2 |
| 2002 | 129 | 8.27 | 41 | 7.23 | 65 | 8.3 |
| 2003 | 132 | 7.93 | 29 | 4.88 | 76 | 8.9 |
| 2004 | 190 | 9.52 | 56 | 8.2 | 104 | 9.6 |
| 2005 | 213 | 9.32 | 50 | 6.07 | 122 | 10. |
| 2006 | 249 | 10.2 | 59 | 7.02 | 147 | 11. |
| 2007 | 340 | 13.3 | 77 | 8.51 | 207 | 15. |
| 2008 | 352 | 13.6 | 91 | 9.48 | 212 | 15. |
| 2009 | 464 | 16.5 | 138 | 12.3 | 256 | 18. |
| 2010 | 479 | 17 | 155 | 13.8 | 270 | 19. |
| 2011 | 544 | 18.9 | 172 | 15.2 | 323 | 21. |
| 2012 | 598 | 20.7 | 200 | 18 | 332 | 22. |
| 2013 | 652 | 23.8 | 211 | 20.2 | 347 | 25 |
| 2014 | 673 | 25 | 278 | 24.1 | 311 | 25 |
| 2015 | 790 | 27.3 | 291 | 24.7 | 378 | 28 |
| 2016 | 820 | 24.7 | 326 | 23.5 | 383 | 24 |
| | | | | | | |

Table 8: Potential precision medicine trials (1995-2016): Generous precision medicine definition for drugs without cancer indications

| | (1) | (2) | (3) | (4) | (5) | (6 |
|------|-------------|---------------------|-------------|--------------------|-------------|------------|
| | PPM Trials: | (/ | PPM Trials: | Share of | PPM Trials: | Shar |
| | All | Share of trials (%) | Phase I | Phase I trials (%) | Phase II | Phase II t |
| 1995 | 4 | .765 | 1 | .481 | 0 | 0 |
| 1996 | 1 | .193 | 0 | 0 | 0 | 0 |
| 1997 | 3 | .407 | 0 | 0 | 3 | 1.1 |
| 1998 | 2 | .238 | 0 | 0 | 1 | .33 |
| 1999 | 8 | .587 | 5 | 1.08 | 2 | .43 |
| 2000 | 9 | .679 | 3 | .775 | 3 | .65 |
| 2001 | 10 | .606 | 2 | .43 | 5 | .8 |
| 2002 | 7 | .324 | 2 | .342 | 2 | .25 |
| 2003 | 24 | .95 | 11 | 1.63 | 5 | .54 |
| 2004 | 30 | .932 | 7 | .837 | 13 | 1.1 |
| 2005 | 37 | .898 | 12 | 1.18 | 13 | .80 |
| 2006 | 38 | .802 | 10 | .763 | 13 | .72 |
| 2007 | 44 | .88 | 22 | 1.52 | 14 | .7 |
| 2008 | 40 | .738 | 18 | 1.05 | 8 | .38 |
| 2009 | 59 | 1.04 | 16 | .857 | 28 | 1.2 |
| 2010 | 54 | .913 | 21 | 1.07 | 22 | .98 |
| 2011 | 70 | 1.15 | 25 | 1.26 | 29 | 1.2 |
| 2012 | 79 | 1.24 | 20 | 1.04 | 40 | 1.4 |
| 2013 | 103 | 1.61 | 32 | 1.6 | 40 | 1.5 |
| 2014 | 123 | 1.82 | 41 | 1.84 | 56 | 1.9 |
| 2015 | 155 | 2.02 | 49 | 2.11 | 72 | 2.0 |
| 2016 | 158 | 1.79 | 42 | 1.58 | 91 | 2.0 |

Table 9: Potential precision medicine trials (1995-2016): Restrictive precision medicine definition for drugs without cancer indications

| | (1) | (2) | (3) | (4) | (5) | (6) |
|------|-------------|------------------------|-------------|--------------------|-------------|-------------|
| | PPM Trials: | | PPM Trials: | Share of | PPM Trials: | Share |
| | All | Share of trials $(\%)$ | Phase I | Phase I trials (%) | Phase II | Phase II to |
| 1995 | 1 | .191 | 0 | 0 | 0 | 0 |
| 1996 | 1 | .193 | 0 | 0 | 0 | 0 |
| 1997 | 3 | .407 | 0 | 0 | 3 | 1.1 |
| 1998 | 1 | .119 | 0 | 0 | 1 | .33 |
| 1999 | 6 | .441 | 3 | .645 | 2 | .43 |
| 2000 | 7 | .528 | 3 | .775 | 2 | .43 |
| 2001 | 5 | .303 | 1 | .215 | 1 | .16 |
| 2002 | 4 | .185 | 0 | 0 | 1 | .12 |
| 2003 | 20 | .792 | 8 | 1.19 | 4 | .43 |
| 2004 | 22 | .683 | 4 | .478 | 8 | .69 |
| 2005 | 27 | .655 | 8 | .784 | 9 | .55 |
| 2006 | 26 | .549 | 5 | .382 | 9 | .50 |
| 2007 | 30 | .6 | 12 | .826 | 11 | .55 |
| 2008 | 28 | .516 | 13 | .76 | 5 | .23 |
| 2009 | 38 | .669 | 10 | .535 | 18 | .79 |
| 2010 | 35 | .592 | 10 | .507 | 15 | .66 |
| 2011 | 48 | .785 | 16 | .808 | 20 | .83 |
| 2012 | 47 | .737 | 9 | .467 | 23 | .85 |
| 2013 | 68 | 1.06 | 20 | 1 | 22 | .83 |
| 2014 | 75 | 1.11 | 28 | 1.25 | 32 | 1.1 |
| 2015 | 93 | 1.21 | 31 | 1.34 | 39 | 1.1 |
| 2016 | 94 | 1.06 | 20 | .754 | 59 | 1.3 |
| | | | | | | |

Table 10: Potential precision medicine trials (1995-2016): Generous precision medicine definition for trials located in US $\,$

| mean | cine deminion for | trials located in US | | | | |
|------|-------------------|----------------------|-------------|--------------------|-------------|-----------------------|
| | (1) | (2) | (3) | (4) | (5) | (6) |
| | PPM Trials: | | PPM Trials: | Share of | PPM Trials: | Shar |
| | All | Share of trials (%) | Phase I | Phase I trials (%) | Phase II | Phase II to |
| 199 | 5 	 10 | 3.8 | 4 | 4.08 | 2 | 1.8 |
| 199 | 6 18 | 4.9 | 4 | 3.25 | 9 | 5.7 |
| 199 | 7 	 24 | 4.41 | 10 | 5.1 | 10 | 4.4 |
| 199 | 8 39 | 5.66 | 6 | 2.7 | 27 | 7.8 |
| 199 | 9 	 52 | 4.2 | 21 | 4.64 | 26 | 5.0 |
| 200 | 0 63 | 5.84 | 18 | 5.22 | 36 | 7.4 |
| 200 | 1 68 | 5.68 | 26 | 6.91 | 32 | 5.7 |
| 200 | 2 91 | 6.12 | 34 | 7.98 | 42 | 6 |
| 200 | 3 104 | 5.95 | 30 | 5.84 | 57 | 7.1 |
| 200 | 4 	 130 | 5.86 | 40 | 5.99 | 74 | 7.4 |
| 200 | 5 	 135 | 5.16 | 36 | 4.58 | 75 | 6.3 |
| 200 | 6 	 177 | 6.03 | 57 | 6 | 96 | 7.4 |
| 200 | 7 230 | 7.52 | 73 | 6.94 | 131 | 9.8 |
| 200 | 8 221 | 6.77 | 71 | 6.05 | 125 | 8.7 |
| 200 | 9 303 | 9.26 | 113 | 9.11 | 145 | 10. |
| 201 | 0 	 285 | 8.89 | 120 | 9.3 | 132 | 10. |
| 201 | 1 322 | 9.9 | 135 | 10.3 | 157 | 12 |
| 201 | 2 336 | 10.7 | 144 | 12 | 153 | 11. |
| 201 | 3 418 | 12.9 | 171 | 13.2 | 178 | 13. |
| 201 | 4 	 445 | 12.8 | 222 | 15.1 | 170 | 12. |
| 201 | 5 530 | 14.5 | 236 | 16.2 | 211 | 14. |
| 201 | 6 491 | 13.7 | 228 | 15.8 | 207 | 13. |
| | | | | | | |

Table 11: Potential precision medicine trials (1995-2016): Restrictive precision medicine definition for trials located in US $\,$

| medicii | ie deminion for | triais located in US | | | | |
|---------|-----------------|----------------------|-------------|--------------------|-------------|-------------|
| | (1) | (2) | (3) | (4) | (5) | (6) |
| | PPM Trials: | | PPM Trials: | Share of | PPM Trials: | Share |
| | All | Share of trials (%) | Phase I | Phase I trials (%) | Phase II | Phase II to |
| 1995 | 9 | 3.42 | 3 | 3.06 | 2 | 1.8 |
| 1996 | 16 | 4.36 | 3 | 2.44 | 8 | 5.1 |
| 1997 | 22 | 4.04 | 9 | 4.59 | 9 | 3.9 |
| 1998 | 37 | 5.37 | 5 | 2.25 | 26 | 7.6 |
| 1999 | 47 | 3.79 | 18 | 3.97 | 24 | 4.6 |
| 2000 | 58 | 5.38 | 16 | 4.64 | 34 | 7 |
| 2001 | 64 | 5.35 | 23 | 6.12 | 31 | 5.5 |
| 2002 | 85 | 5.72 | 31 | 7.28 | 40 | 5.7 |
| 2003 | 94 | 5.37 | 24 | 4.67 | 53 | 6.6 |
| 2004 | 120 | 5.41 | 35 | 5.24 | 69 | 6.9 |
| 2005 | 120 | 4.59 | 30 | 3.82 | 68 | 5.7 |
| 2006 | 156 | 5.31 | 49 | 5.16 | 85 | 6.6 |
| 2007 | 208 | 6.8 | 60 | 5.7 | 123 | 9.2 |
| 2008 | 206 | 6.31 | 64 | 5.45 | 118 | 8.3 |
| 2009 | 264 | 8.07 | 93 | 7.49 | 130 | 9.1 |
| 2010 | 262 | 8.17 | 111 | 8.6 | 119 | 9.2 |
| 2011 | 298 | 9.16 | 121 | 9.24 | 150 | 11. |
| 2012 | 304 | 9.68 | 128 | 10.6 | 140 | 10. |
| 2013 | 383 | 11.8 | 156 | 12 | 164 | 12. |
| 2014 | 393 | 11.3 | 200 | 13.6 | 146 | 10. |
| 2015 | 465 | 12.7 | 212 | 14.5 | 179 | 12 |
| 2016 | 415 | 11.6 | 197 | 13.6 | 170 | 11. |
| | | | | | | |

Table 12: Potential precision medicine trials (1995-2016): Generous precision medicine definition for trials located outside US $\,$

| medicin | <u>ie dennition for</u> | trials located outside | | | | |
|---------|-------------------------|------------------------|-------------|--------------------|-------------|-------------|
| | (1) | (2) | (3) | (4) | (5) | (6) |
| | PPM Trials: | | PPM Trials: | Share of | PPM Trials: | Share |
| - | All | Share of trials (%) | Phase I | Phase I trials (%) | Phase II | Phase II to |
| 1995 | 1 | .625 | 0 | 0 | 0 | 0 |
| 1996 | 4 | 2.34 | 1 | 2.86 | 0 | 0 |
| 1997 | 7 | 2.72 | 0 | 0 | 3 | 3.9 |
| 1998 | 12 | 4.15 | 2 | 3.51 | 2 | 2.2 |
| 1999 | 13 | 3.64 | 2 | 2.63 | 4 | 3.3 |
| 2000 | 16 | 3.33 | 3 | 2.78 | 6 | 3.4 |
| 2001 | 29 | 4.26 | 8 | 5.19 | 9 | 3.5 |
| 2002 | 34 | 3.63 | 4 | 2.03 | 18 | 4.8 |
| 2003 | 49 | 4.03 | 13 | 4.85 | 20 | 4.0 |
| 2004 | 81 | 4.56 | 18 | 5.68 | 41 | 5.1 |
| 2005 | 106 | 4.23 | 26 | 5.12 | 53 | 4.6 |
| 2006 | 103 | 3.33 | 14 | 2.14 | 58 | 4.0 |
| 2007 | 152 | 4.41 | 28 | 3.62 | 85 | 5.2 |
| 2008 | 169 | 4.4 | 37 | 3.65 | 96 | 5.5 |
| 2009 | 237 | 5.35 | 59 | 4.52 | 143 | 7.2 |
| 2010 | 265 | 5.44 | 63 | 4.46 | 174 | 7.9 |
| 2011 | 296 | 5.85 | 68 | 4.69 | 191 | 8.1 |
| 2012 | 340 | 6.25 | 78 | 5.18 | 218 | 8.3 |
| 2013 | 347 | 6.4 | 82 | 5.2 | 207 | 8.2 |
| 2014 | 367 | 6.68 | 109 | 6.4 | 202 | 7.8 |
| 2015 | 445 | 7.03 | 115 | 6.31 | 260 | 8.3 |
| 2016 | 501 | 6.58 | 171 | 7.52 | 259 | 6.5 |
| | | | | | | |

Table 13: Potential precision medicine trials (1995-2016): Restrictive precision medicine definition for trials located outside US $\,$

| medicin | <u>ie dennition for</u> | trials located outside | | | | |
|---------|-------------------------|------------------------|-------------|--------------------|-------------|-------------|
| | (1) | (2) | (3) | (4) | (5) | (6) |
| | PPM Trials: | | PPM Trials: | Share of | PPM Trials: | Share |
| - | All | Share of trials (%) | Phase I | Phase I trials (%) | Phase II | Phase II to |
| 1995 | 0 | 0 | 0 | 0 | 0 | 0 |
| 1996 | 4 | 2.34 | 1 | 2.86 | 0 | 0 |
| 1997 | 7 | 2.72 | 0 | 0 | 3 | 3.9 |
| 1998 | 11 | 3.81 | 2 | 3.51 | 2 | 2.2 |
| 1999 | 13 | 3.64 | 2 | 2.63 | 4 | 3.3 |
| 2000 | 16 | 3.33 | 3 | 2.78 | 6 | 3.4 |
| 2001 | 26 | 3.82 | 6 | 3.9 | 8 | 3.1 |
| 2002 | 31 | 3.31 | 4 | 2.03 | 16 | 4.2 |
| 2003 | 46 | 3.78 | 12 | 4.48 | 19 | 3.8 |
| 2004 | 71 | 4 | 16 | 5.05 | 33 | 4.1 |
| 2005 | 99 | 3.95 | 23 | 4.53 | 49 | 4.3 |
| 2006 | 101 | 3.26 | 13 | 1.99 | 58 | 4.0 |
| 2007 | 139 | 4.03 | 21 | 2.72 | 81 | 4.9 |
| 2008 | 157 | 4.09 | 32 | 3.16 | 91 | 5.2 |
| 2009 | 217 | 4.9 | 50 | 3.83 | 133 | 6.7 |
| 2010 | 241 | 4.95 | 54 | 3.82 | 161 | 7.3 |
| 2011 | 273 | 5.4 | 58 | 4 | 181 | 7.7 |
| 2012 | 322 | 5.92 | 74 | 4.91 | 206 | 7.9 |
| 2013 | 321 | 5.92 | 71 | 4.51 | 194 | 7.7 |
| 2014 | 334 | 6.08 | 101 | 5.93 | 183 | 7.0 |
| 2015 | 388 | 6.13 | 95 | 5.21 | 228 | 7.2 |
| 2016 | 442 | 5.81 | 136 | 5.98 | 239 | 6.0 |
| | | | | | | |

Table 14: Share of trials receiving NIH funding: Generous precision medicine $\underline{\text{definition}}$

| | (1) | (2) | (3) | (4) | (5) | (6) |
|------------------|----------|-----------|------------|--------------|--------------|-------------|
| | Phase I: | Phase II: | Phase III: | Phase I: | Phase II: | Phase III: |
| | All | All | All | Generous PPM | Generous PPM | Generous PF |
| Share funded (%) | | | | | | |
| 1995 | 2.10 | 5.99 | 3.26 | 0.00 | 100.00 | 0.00 |
| 1996 | 3.54 | 4.81 | 3.39 | 0.00 | 10.00 | 11.11 |
| 1997 | 5.56 | 8.02 | 3.05 | 9.09 | 6.25 | 0.00 |
| 1998 | 5.34 | 6.00 | 1.89 | 0.00 | 19.35 | 6.67 |
| 1999 | 4.08 | 6.93 | 2.95 | 15.38 | 8.11 | 0.00 |
| 2000 | 7.29 | 6.10 | 2.64 | 7.41 | 10.42 | 10.00 |
| 2001 | 4.73 | 5.67 | 3.27 | 14.63 | 12.00 | 0.00 |
| 2002 | 3.91 | 4.97 | 2.51 | 15.22 | 12.86 | 3.57 |
| 2003 | 5.13 | 4.14 | 2.07 | 6.67 | 5.88 | 2.78 |
| 2004 | 4.08 | 5.81 | 1.15 | 16.18 | 7.94 | 2.50 |
| 2005 | 4.34 | 4.94 | 1.60 | 4.48 | 9.79 | 3.77 |
| 2006 | 4.05 | 4.75 | 0.98 | 12.16 | 9.58 | 1.72 |
| 2007 | 3.22 | 3.73 | 0.75 | 7.34 | 9.96 | 0.00 |
| 2008 | 3.29 | 3.62 | 0.88 | 17.24 | 10.92 | 4.76 |
| 2009 | 3.54 | 3.18 | 0.81 | 8.43 | 6.00 | 0.00 |
| 2010 | 2.74 | 2.47 | 0.75 | 11.35 | 4.82 | 1.49 |
| 2011 | 1.96 | 2.43 | 0.69 | 5.14 | 5.26 | 0.00 |
| 2012 | 2.07 | 2.04 | 0.24 | 5.63 | 4.46 | 0.00 |
| 2013 | 2.66 | 2.81 | 0.34 | 5.84 | 7.32 | 0.78 |
| 2014 | 3.13 | 2.25 | 0.52 | 5.04 | 3.87 | 0.00 |
| 2015 | 2.63 | 2.48 | 0.82 | 6.52 | 6.64 | 0.63 |
| 2016 | 2.67 | 1.85 | 0.64 | 6.47 | 4.79 | 0.00 |
| \overline{N} | 43615 | 57636 | 30720 | 2837 | 4365 | 1283 |
| | | | | | | |

Table 15: Number of US trials employing biomarkers by type

| | able 15. Nulliber | of O5 thats employing | ng biomarkers | 0 01 | | | |
|---------|-------------------|-----------------------|---------------|----------|-----------|---------------|-----|
| | Any biomarker | Anthropomorphic | Biochemical | Cellular | Genomic | Physiological | Pro |
| Overall | 22,881 | 1,981 | 6,442 | 2,102 | 14,093 | 4,284 | 14 |
| 1995 | 54 | 2 | 11 | 0 | 30 | 11 | |
| 1996 | 76 | 4 | 14 | 5 | 45 | 7 | |
| 1997 | 103 | 4 | 26 | 4 | 66 | 11 | |
| 1998 | 139 | 6 | 35 | 2 | 94 | 15 | |
| 1999 | 261 | 8 | 54 | 18 | 192 | 17 | |
| 2000 | 293 | 23 | 74 | 16 | 211 | 36 | |
| 2001 | 328 | 18 | 82 | 22 | 235 | 38 | |
| 2002 | 472 | 29 | 131 | 17 | 327 | 60 | ; |
| 2003 | 551 | 45 | 167 | 30 | 362 | 74 | ; |
| 2004 | 762 | 63 | 208 | 40 | 491 | 88 | ļ |
| 2005 | 940 | 73 | 280 | 73 | 601 | 147 | (|
| 2006 | 1,090 | 83 | 332 | 82 | 703 | 175 | , |
| 2007 | 1,260 | 118 | 362 | 122 | 847 | 177 | 6 |
| 2008 | 1,414 | 129 | 461 | 145 | 896 | 227 | 9 |
| 2009 | 1,531 | 134 | 482 | 141 | 995 | 260 | 1 |
| 2010 | 1,721 | 165 | 510 | 189 | 1,075 | 308 | 1 |
| 2011 | 1,811 | 151 | 552 | 176 | $1,\!171$ | 342 | 1 |
| 2012 | 1,809 | 172 | 563 | 211 | 1,081 | 409 | 1 |
| 2013 | 1,917 | 180 | 565 | 210 | $1,\!121$ | 441 | 1 |
| 2014 | 2,070 | 207 | 574 | 221 | 1,158 | 503 | 1 |
| 2015 | 2,203 | 207 | 482 | 196 | 1,232 | 510 | 1 |
| 2016 | 2,076 | 160 | 477 | 182 | 1,160 | 428 | 1 |

Table 16: Number of US trials employing biomarkers by detailed role

| | 10. Number | Table 10: Number of US trials employing biomarkers by detailed role | | | | | | | |
|---------|------------|---|--------------|-----------------------|------------|------------|-----------|------|--|
| | | | | Predicting | Predicting | Predicting | | Sele | |
| | Any | | Differential | drug | treatment | treatment | | fe | |
| | biomarker | Diagnosis | Diagnosis | resistance | efficacy | toxicity | Screening | the | |
| Overall | 17,118 | 15,095 | 8,936 | 3,406 | $13,\!396$ | $2,\!158$ | 2,393 | 4,8 | |
| 1995 | 54 | 35 | 23 | 5 | 33 | 4 | 5 | | |
| 1996 | 76 | 44 | 22 | 13 | 42 | 4 | 5 | | |
| 1997 | 103 | 64 | 38 | 20 | 61 | 15 | 19 | 2 | |
| 1998 | 139 | 95 | 50 | 33 | 91 | 18 | 18 | 2 | |
| 1999 | 261 | 187 | 86 | 45 | 172 | 26 | 25 | 4 | |
| 2000 | 293 | 190 | 103 | 37 | 176 | 25 | 22 | 4 | |
| 2001 | 328 | 221 | 113 | 56 | 207 | 29 | 33 | 6 | |
| 2002 | 472 | 340 | 196 | 86 | 318 | 61 | 55 | 1 | |
| 2003 | 551 | 375 | 236 | 100 | 343 | 66 | 68 | 1 | |
| 2004 | 762 | 518 | 311 | 134 | 480 | 99 | 89 | 1 | |
| 2005 | 940 | 636 | 380 | 158 | 574 | 109 | 109 | 1 | |
| 2006 | 1,090 | 767 | 459 | 191 | 667 | 121 | 127 | 2 | |
| 2007 | 1,260 | 883 | 529 | 218 | 802 | 135 | 141 | 2 | |
| 2008 | 1,414 | 968 | 626 | 247 | 874 | 178 | 162 | 3 | |
| 2009 | 1,531 | 1,077 | 702 | 306 | 984 | 207 | 201 | 4 | |
| 2010 | 1,721 | 1,130 | 648 | 274 | 1,010 | 143 | 179 | 3 | |
| 2011 | 1,811 | 1,205 | 723 | 275 | 1,066 | 160 | 197 | 3 | |
| 2012 | 1,809 | 1,200 | 711 | 236 | 1,051 | 150 | 157 | 3 | |
| 2013 | 1,917 | 1,221 | 710 | 274 | 1,092 | 160 | 204 | 4 | |
| 2014 | 2,070 | 1,295 | 739 | 240 | 1,105 | 155 | 188 | 4 | |
| 2015 | 2,203 | 1,337 | 774 | 237 | 1,142 | 147 | 205 | 4 | |
| 2016 | 2,076 | 1,307 | 757 | 221 | 1,106 | 146 | 184 | 4 | |

Table 17: US potential precision medicine trials (1995-2016): Generous precision medicine definition for drugs with cancer indications

| PPM Trials: All Share of trials (%) PPM Trials: Phase I Share of Phase I trials (%) PPM Trials: Phase II Phase II Phase II 1995 8 6.15 3 5.36 2 1996 18 8.57 4 5.19 9 1997 23 7.82 10 7.94 9 1998 38 8.15 6 3.66 26 1999 45 8.09 16 7.55 24 2000 57 8.78 15 6.1 34 2001 65 10.1 24 10.2 31 2002 85 12.3 32 12.8 41 2003 91 11.8 22 8.21 56 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 | | (1) | (2) | (3) | (4) | (5) | (6 |
|---|------|-----|---------------------|-----|----------|-----|------------|
| All Share of trials (%) Phase I Phase I trials (%) Phase II Phase II 1995 8 6.15 3 5.36 2 1996 18 8.57 4 5.19 9 1997 23 7.82 10 7.94 9 1998 38 8.15 6 3.66 26 1999 45 8.09 16 7.55 24 2000 57 8.78 15 6.1 34 2001 65 10.1 24 10.2 31 2002 85 12.3 32 12.8 41 2003 91 11.8 22 8.21 56 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 | | | (-) | | Share of | | Shar |
| 1996 18 8.57 4 5.19 9 1997 23 7.82 10 7.94 9 1998 38 8.15 6 3.66 26 1999 45 8.09 16 7.55 24 2000 57 8.78 15 6.1 34 2001 65 10.1 24 10.2 31 2002 85 12.3 32 12.8 41 2003 91 11.8 22 8.21 56 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 | | | Share of trials (%) | | | | Phase II t |
| 1997 23 7.82 10 7.94 9 1998 38 8.15 6 3.66 26 1999 45 8.09 16 7.55 24 2000 57 8.78 15 6.1 34 2001 65 10.1 24 10.2 31 2002 85 12.3 32 12.8 41 2003 91 11.8 22 8.21 56 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 | 1995 | 8 | 6.15 | 3 | 5.36 | 2 | 4 |
| 1998 38 8.15 6 3.66 26 1999 45 8.09 16 7.55 24 2000 57 8.78 15 6.1 34 2001 65 10.1 24 10.2 31 2002 85 12.3 32 12.8 41 2003 91 11.8 22 8.21 56 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2014 | 1996 | 18 | 8.57 | 4 | 5.19 | 9 | 10 |
| 1999 45 8.09 16 7.55 24 2000 57 8.78 15 6.1 34 2001 65 10.1 24 10.2 31 2002 85 12.3 32 12.8 41 2003 91 11.8 22 8.21 56 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014< | 1997 | 23 | 7.82 | 10 | 7.94 | 9 | 6.5 |
| 2000 57 8.78 15 6.1 34 2001 65 10.1 24 10.2 31 2002 85 12.3 32 12.8 41 2003 91 11.8 22 8.21 56 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 20 | 1998 | 38 | 8.15 | 6 | 3.66 | 26 | 10. |
| 2001 65 10.1 24 10.2 31 2002 85 12.3 32 12.8 41 2003 91 11.8 22 8.21 56 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 <td>1999</td> <td>45</td> <td>8.09</td> <td>16</td> <td>7.55</td> <td>24</td> <td>8.5</td> | 1999 | 45 | 8.09 | 16 | 7.55 | 24 | 8.5 |
| 2002 85 12.3 32 12.8 41 2003 91 11.8 22 8.21 56 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2000 | 57 | 8.78 | 15 | 6.1 | 34 | 10. |
| 2003 91 11.8 22 8.21 56 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2001 | 65 | 10.1 | 24 | 10.2 | 31 | 9.0 |
| 2004 117 11.8 38 10.9 69 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2002 | 85 | 12.3 | 32 | 12.8 | 41 | 10. |
| 2005 119 11 31 7.33 70 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2003 | 91 | 11.8 | 22 | 8.21 | 56 | 13. |
| 2006 155 13.3 48 10.4 90 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2004 | 117 | 11.8 | 38 | 10.9 | 69 | 12. |
| 2007 201 16.7 56 11.2 125 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2005 | 119 | 11 | 31 | 7.33 | 70 | 12. |
| 2008 207 18 65 12.7 120 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2006 | 155 | 13.3 | 48 | 10.4 | 90 | 14. |
| 2009 271 22.2 106 18.3 129 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2007 | 201 | 16.7 | 56 | 11.2 | 125 | 20. |
| 2010 257 21.1 112 18.8 121 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2008 | 207 | 18 | 65 | 12.7 | 120 | 21. |
| 2011 290 24.8 125 22 143 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2009 | 271 | 22.2 | 106 | 18.3 | 129 | 24. |
| 2012 293 26.1 132 23.1 132 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2010 | 257 | 21.1 | 112 | 18.8 | 121 | 23. |
| 2013 354 30.8 153 26.8 156 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2011 | 290 | 24.8 | 125 | 22 | 143 | 27. |
| 2014 387 31.9 202 30.8 143 2015 453 34.2 212 31.3 180 | 2012 | 293 | 26.1 | 132 | 23.1 | 132 | 28. |
| 2015 453 34.2 212 31.3 180 | 2013 | 354 | 30.8 | 153 | 26.8 | 156 | 31. |
| | 2014 | 387 | 31.9 | 202 | 30.8 | 143 | 30. |
| 2016 413 30.9 205 29.5 165 | 2015 | 453 | 34.2 | 212 | 31.3 | 180 | 33. |
| 2010 110 90.5 200 20.0 100 | 2016 | 413 | 30.9 | 205 | 29.5 | 165 | 31 |

Table 18: US potential precision medicine trials (1995-2016): Restrictive precision medicine definition for drugs with cancer indications

| | | on for druge with come | | | | |
|------|-------------|------------------------|-------------|--------------------|-------------|------------|
| | (1) | (2) | (3) | (4) | (5) | \sim (6) |
| | PPM Trials: | ~; | PPM Trials: | Share of | PPM Trials: | Shar |
| | All | Share of trials (%) | Phase I | Phase I trials (%) | Phase II | Phase II t |
| 1995 | 8 | 6.15 | 3 | 5.36 | 2 | 4 |
| 1996 | 16 | 7.62 | 3 | 3.9 | 8 | 8.8 |
| 1997 | 21 | 7.14 | 9 | 7.14 | 8 | 5.8 |
| 1998 | 36 | 7.73 | 5 | 3.05 | 25 | 9.8 |
| 1999 | 42 | 7.55 | 15 | 7.08 | 22 | 7.8 |
| 2000 | 54 | 8.32 | 13 | 5.28 | 33 | 10. |
| 2001 | 63 | 9.75 | 22 | 9.36 | 31 | 9.0 |
| 2002 | 81 | 11.7 | 31 | 12.4 | 39 | 10. |
| 2003 | 84 | 10.9 | 19 | 7.09 | 52 | 12. |
| 2004 | 110 | 11.1 | 35 | 10.1 | 65 | 11. |
| 2005 | 111 | 10.2 | 28 | 6.62 | 65 | 11. |
| 2006 | 144 | 12.4 | 44 | 9.54 | 83 | 13 |
| 2007 | 189 | 15.7 | 52 | 10.4 | 118 | 19 |
| 2008 | 198 | 17.2 | 62 | 12.1 | 114 | 20 |
| 2009 | 245 | 20 | 89 | 15.4 | 120 | 22 |
| 2010 | 242 | 19.9 | 106 | 17.8 | 112 | 21 |
| 2011 | 274 | 23.4 | 115 | 20.3 | 139 | 2 |
| 2012 | 279 | 24.8 | 123 | 21.5 | 128 | 27 |
| 2013 | 340 | 29.6 | 146 | 25.6 | 152 | 31 |
| 2014 | 363 | 30 | 188 | 28.7 | 133 | 28 |
| 2015 | 415 | 31.3 | 196 | 29 | 161 | 30 |
| 2016 | 376 | 28.1 | 186 | 26.8 | 149 | 28 |
| | | | | | | |

Table 19: US potential precision medicine trials (1995-2016): Generous precision medicine definition for drugs without cancer indications

| | (1) | (2) | (3) | (4) | (5) | (6) |
|------|-------------|---------------------|-------------|--------------------|-------------|-------------|
| | PPM Trials: | (04) | PPM Trials: | Share of | PPM Trials: | Share |
| | All | Share of trials (%) | Phase I | Phase I trials (%) | Phase II | Phase II to |
| 1995 | 2 | 1.5 | 1 | 2.38 | 0 | 0 |
| 1996 | 0 | 0 | 0 | 0 | 0 | 0 |
| 1997 | 1 | .4 | 0 | 0 | 1 | 1.1 |
| 1998 | 1 | .448 | 0 | 0 | 1 | 1.1 |
| 1999 | 7 | 1.02 | 5 | 2.07 | 2 | .84 |
| 2000 | 6 | 1.4 | 3 | 3.03 | 2 | 1.1 |
| 2001 | 3 | .544 | 2 | 1.42 | 1 | .45 |
| 2002 | 6 | .754 | 2 | 1.14 | 1 | .31 |
| 2003 | 13 | 1.33 | 8 | 3.25 | 1 | .26 |
| 2004 | 13 | 1.06 | 2 | .625 | 5 | 1.1 |
| 2005 | 16 | 1.05 | 5 | 1.38 | 5 | .83 |
| 2006 | 22 | 1.24 | 9 | 1.84 | 6 | .89 |
| 2007 | 29 | 1.56 | 17 | 3.07 | 6 | .81 |
| 2008 | 14 | .662 | 6 | .906 | 5 | .57 |
| 2009 | 32 | 1.56 | 7 | 1.06 | 16 | 1.8 |
| 2010 | 28 | 1.41 | 8 | 1.15 | 11 | 1.4 |
| 2011 | 32 | 1.54 | 10 | 1.35 | 14 | 1.7 |
| 2012 | 43 | 2.13 | 12 | 1.9 | 21 | 2.4 |
| 2013 | 64 | 3.05 | 18 | 2.48 | 22 | 2.6 |
| 2014 | 58 | 2.57 | 20 | 2.45 | 27 | 3.0 |
| 2015 | 77 | 3.3 | 24 | 3.07 | 31 | 3.2 |
| 2016 | 78 | 3.46 | 23 | 3.07 | 42 | 4.2 |
| | | | | | | |

Table 20: US potential precision medicine trials (1995-2016): Restrictive precision medicine definition for drugs without cancer indications

| PPM Trials: All Share of trials (%) PPM Trials: Phase I Share of Phase I trials (%) PPM Trials: Phase II trials (%) Phase II trials (%) PPM Trials: Phase II trials (%) Phase II trials (%) Phase II trials (%) O 1996 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 0 1 1.1 1.1 1.1 1.1 1.1 1.1 1.1 1.1 1.1 1.1 1.1 1.2 2.8 2.8 2.8 2.8 2.8 2.8 2.8 2.8 2.8 2.8 2.8 2.0 3.0 1.1 2.0 2.2 2.2 2.2 2.2 2.2 2.2< | | | | | | | |
|---|------|-----|------|-----|--------------------|----------|-------------|
| All Share of trials (%) Phase I Phase I trials (%) Phase II Phase II trials (%) 1995 1 .752 0 0 0 0 1996 0 0 0 0 0 0 1997 1 .4 0 0 1 1.1 1998 1 .448 0 0 1 1.1 1999 5 .732 3 1.24 2 .84 2000 4 .93 3 3.03 1 .59 2001 1 .181 1 .709 0 0 2002 4 .503 0 0 1 .31 2003 10 1.03 5 2.03 1 .26 2004 10 .812 0 0 4 .89 2005 9 .589 2 .551 3 .49 2006 12 .678 | | | (2) | (3) | | (5) | |
| 1995 1 .752 0 0 0 0 0 1996 0 0 0 0 0 0 0 1997 1 .4 0 0 1 1.1 1.1 1998 1 .448 0 0 1 1.1 1.1 1.1 1.999 5 .732 3 1.24 2 .84 2000 4 .93 3 3.03 1 .59 2000 4 .93 3 3.03 1 .59 2001 1 .181 1 .709 0 4 .89 2003 1 .26 2004 1 .89 2005 9 .589 2 .551 3 .49 2005 9 .589 2 <td></td> <td></td> <td></td> <td></td> <td></td> <td></td> <td></td> | | | | | | | |
| $\begin{array}{cccccccccccccccccccccccccccccccccccc$ | | All | | | Phase I trials (%) | Phase II | Phase II to |
| $\begin{array}{cccccccccccccccccccccccccccccccccccc$ | 1995 | 1 | .752 | 0 | 0 | 0 | 0 |
| $\begin{array}{cccccccccccccccccccccccccccccccccccc$ | 1996 | 0 | 0 | 0 | 0 | 0 | 0 |
| 1999 5 .732 3 1.24 2 .84 2000 4 .93 3 3.03 1 .59 2001 1 .181 1 .709 0 0 2002 4 .503 0 0 1 .31 2003 10 1.03 5 2.03 1 .26 2004 10 .812 0 0 4 .89 2005 9 .589 2 .551 3 .49 2005 9 .589 2 .551 3 .49 2006 12 .678 5 1.02 2 .29 2007 19 1.02 8 1.45 5 .68 2008 8 .378 2 .302 4 .46 2009 19 .928 4 .603 10 1.1 2010 20 1.01 5 .719 7 .91 2011 24 1.15 6 .809< | 1997 | 1 | .4 | 0 | 0 | 1 | 1.1 |
| 2000 4 .93 3 3.03 1 .59 2001 1 .181 1 .709 0 0 2002 4 .503 0 0 1 .31 2003 10 1.03 5 2.03 1 .26 2004 10 .812 0 0 4 .89 2005 9 .589 2 .551 3 .49 2005 9 .589 2 .551 3 .49 2006 12 .678 5 1.02 2 .29 2007 19 1.02 8 1.45 5 .68 2008 8 .378 2 .302 4 .46 2009 19 .928 4 .603 10 1.1 2010 20 1.01 5 .719 7 .91 2011 24 1.15 6 <td>1998</td> <td>1</td> <td>.448</td> <td>0</td> <td>0</td> <td>1</td> <td>1.1</td> | 1998 | 1 | .448 | 0 | 0 | 1 | 1.1 |
| $\begin{array}{c ccccccccccccccccccccccccccccccccccc$ | 1999 | 5 | .732 | 3 | 1.24 | 2 | .84 |
| $\begin{array}{cccccccccccccccccccccccccccccccccccc$ | 2000 | 4 | .93 | 3 | 3.03 | 1 | .59 |
| 2003 10 1.03 5 2.03 1 .26 2004 10 .812 0 0 4 .89 2005 9 .589 2 .551 3 .49 2006 12 .678 5 1.02 2 .29 2007 19 1.02 8 1.45 5 .68 2008 8 .378 2 .302 4 .46 2009 19 .928 4 .603 10 1.1 2010 20 1.01 5 .719 7 .91 2011 24 1.15 6 .809 11 1.3 2012 25 1.24 5 .794 12 1.4 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.5 | 2001 | 1 | .181 | 1 | .709 | 0 | 0 |
| 2004 10 .812 0 0 4 .89 2005 9 .589 2 .551 3 .49 2006 12 .678 5 1.02 2 .29 2007 19 1.02 8 1.45 5 .68 2008 8 .378 2 .302 4 .46 2009 19 .928 4 .603 10 1.1 2010 20 1.01 5 .719 7 .91 2011 24 1.15 6 .809 11 1.3 2012 25 1.24 5 .794 12 1.4 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.5 | 2002 | 4 | .503 | 0 | 0 | 1 | .31 |
| 2005 9 .589 2 .551 3 .49 2006 12 .678 5 1.02 2 .29 2007 19 1.02 8 1.45 5 .68 2008 8 .378 2 .302 4 .46 2009 19 .928 4 .603 10 1.1 2010 20 1.01 5 .719 7 .91 2011 24 1.15 6 .809 11 1.3 2012 25 1.24 5 .794 12 1.4 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.5 | 2003 | 10 | 1.03 | 5 | 2.03 | 1 | .26 |
| 2006 12 .678 5 1.02 2 .29 2007 19 1.02 8 1.45 5 .68 2008 8 .378 2 .302 4 .46 2009 19 .928 4 .603 10 1.1 2010 20 1.01 5 .719 7 .91 2011 24 1.15 6 .809 11 1.3 2012 25 1.24 5 .794 12 1.4 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.5 | 2004 | 10 | .812 | 0 | 0 | 4 | .89 |
| 2007 19 1.02 8 1.45 5 .68 2008 8 .378 2 .302 4 .46 2009 19 .928 4 .603 10 1.1 2010 20 1.01 5 .719 7 .91 2011 24 1.15 6 .809 11 1.3 2012 25 1.24 5 .794 12 1.4 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.9 | 2005 | 9 | .589 | 2 | .551 | 3 | .49 |
| 2008 8 .378 2 .302 4 .46 2009 19 .928 4 .603 10 1.1 2010 20 1.01 5 .719 7 .91 2011 24 1.15 6 .809 11 1.3 2012 25 1.24 5 .794 12 1.4 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.5 | 2006 | 12 | .678 | 5 | 1.02 | 2 | .29 |
| 2009 19 .928 4 .603 10 1.1 2010 20 1.01 5 .719 7 .91 2011 24 1.15 6 .809 11 1.3 2012 25 1.24 5 .794 12 1.4 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.5 | 2007 | 19 | 1.02 | 8 | 1.45 | 5 | .68 |
| 2010 20 1.01 5 .719 7 .91 2011 24 1.15 6 .809 11 1.3 2012 25 1.24 5 .794 12 1.4 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.9 | 2008 | 8 | .378 | 2 | .302 | 4 | .46 |
| 2011 24 1.15 6 .809 11 1.3 2012 25 1.24 5 .794 12 1.4 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.9 | 2009 | 19 | .928 | 4 | .603 | 10 | 1.1 |
| 2012 25 1.24 5 .794 12 1.4 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.9 | 2010 | 20 | 1.01 | 5 | .719 | 7 | .91 |
| 2013 43 2.05 10 1.38 12 1.4 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.9 | 2011 | 24 | 1.15 | 6 | .809 | 11 | 1.3 |
| 2014 30 1.33 12 1.47 13 1.4 2015 50 2.14 16 2.04 18 1.9 | 2012 | 25 | 1.24 | 5 | .794 | 12 | 1.4 |
| 2015 50 2.14 16 2.04 18 1.9 | 2013 | 43 | 2.05 | 10 | 1.38 | 12 | 1.4 |
| 2015 50 2.14 16 2.04 18 1.9 | 2014 | 30 | 1.33 | 12 | 1.47 | 13 | 1.4 |
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