Where applicable for your device, you should describe the quality control design specifications in place to address the following:

- Correct placement and identity of instrument features (e.g., probes).
- For multiplexed tests in which the target molecules will contact a number of different probes, the potential for specific and non-specific probe cross-hybridization.
- Prevention of probe cross-contamination, for multiplexed tests in which many probes are handled during the manufacturing process.

Illustrations or photographs of non-standard equipment or methods can be helpful in understanding novel methodologies.

Test Algorithms

Because of the large number of polymorphisms found within some DME genes, and the fact that some polymorphisms may be common to various alleles, you should explain how the test algorithms were developed to report DME genotype, if applicable.

Test Results

You should provide examples of the test reports (e.g., printouts) that are generated for the clinician. These reports should be consistent with current recommendations of genetics professional societies, if applicable, and should contain adequate interpretation guidelines for the use of the ordering physician/counselor.

Where applicable, reports should describe the polymorphisms identified by your test and the methodology and technology used for detection. You should identify representative literature references describing genotypic interpretations, or phenotypic predictions, if applicable, to enable users to access information about specific genotypes. We expect that for most 510(k) applications, appropriate literature references will be available. If such supporting information is not available, you should provide information based on the clinical studies you conducted to address clinical validity of your device.

7. Performance Characteristics

In your 510(k), you should detail the study design you used to evaluate each of the performance characteristics outlined below. This should include information such as:

- Description of the samples you used in testing (including types of samples, preparation or origin, number of samples and how the samples specifically represent your intended use samples).
- Descriptions of the computations and statistical analyses you used to evaluate your data.
- Explanations, if there were any deviations from your protocol.

For each of the performance characteristics described below, you should also provide a clear description of all results and acceptance criteria in your 510(k).

The issues described below generally apply, regardless of the technology used by the device to detect the DME genotype. If you make additional claims for other performance characteristics not mentioned below, you should describe the study designs and results you used to support them.

Preanalytical Factors

Consideration of preanalytical factors is critical for high-quality genetic tests. If you intend to provide reagents for extraction and preparation of DNA for testing, you should validate each step in the preanalytical process for its effects on reproducibility, accuracy, and stability of product and describe study design and results addressing this issue in the 510(k). Your external site studies (e.g., reproducibility, method comparison) should include evaluation of the preanalytical process.

If you do not intend to provide reagents for DNA extraction and preparation as part of the assay, you should provide specifications for the reagents needed and for assessing the quality of the assay input DNA, so that the user can select appropriate reagents. You should describe, in your 510(k), the study design and results you used to determine these specifications. In this case, we recommend that in studies establishing the performance of your device, you permit sites to use any extraction method they choose, provided that it meets your test's specifications. In this way, you can evaluate any effect of variations in preanalytical processes on your device performance.

You should evaluate the accuracy and precision of your assay, including DNA extraction, from all the sources of DNA that you recommend for your assay (e.g., blood, PBMC, buccal swab). You should also evaluate all sample collection and storage options you recommend (e.g., heparin-preserved vs. EDTA-preserved blood, stored vs. fresh sample). Your validation of appropriate storage conditions should include both the sample and the extracted product.

You should evaluate the stability of all of your reagents and recommended samples.

Quality Control

DME genotyping systems should include both positive and negative controls. Controls should approximate sample composition and DNA concentration in order to adequately challenge the system.

You should describe the following concerning quality control and calibration material:

- The nature and the function of the various controls that you include with, or recommend for, your system. These controls may differ between individual technologies, but they should enable the user to determine if all steps and critical reactions have proceeded properly and without contamination or cross-hybridization.
- Your methods for value assignment and validation of control and calibrator material.

- Your methods for establishing quality control and calibration procedures, including your recommended frequency.

We recommend that you provide for the calibration of DME genotyping systems where appropriate.

Analytical Factors

Analytical Sensitivity and Assay Limits

You should validate the analytical sensitivity of your test. This may be defined as the lowest amount of genomic DNA for which the assay can detect genotypes with a given accuracy and precision. You should also approximate the volume of the clinical sample required to generate this minimum input. We recommend that you determine analytical sensitivity using samples containing genomic DNA at varying concentrations. You should test a statistically determined number of replicates at each DNA level. Similarly, you should determine the upper limit of the assay, in terms of DNA concentration and sample volume.

Interference

Where applicable, you should evaluate cross-contamination of your device. In particular, you should perform studies to characterize potential carryover by alternating specimens of known genotype. You should also evaluate homologous gene sequences for cross-reactivity.

Potential interfering substances may not always be removed by sample preparation, and may also interfere with sample preparation. Therefore, we recommend that you characterize the effects of potential interfering substances on assay performance. Examples of experimental designs, including guidelines for selecting interfering substances for testing, are described in detail in "Interference Testing in Clinical Chemistry; Approved Guideline" (NCCLS document EP7-A, 2002). Potential interfering substances can include compounds normally found in serum, such as triglycerides, hemoglobin (for specimens other than blood), bilirubin, lipids, and exogenous compounds such as common drugs.

Precision (Repeatability/Reproducibility)

You should fully examine the reproducibility of your DME genotyping system. "Evaluation of Precision Performance of Clinical Chemistry Devices" (NCCLS Document EP5-A) and "User Protocol for Evaluation of Qualitative Test Performance" (NCCLS EP-12A) include guidelines for experimental design, computations, and a format for stating performance claims. We recommend that you incorporate the following in the design of your reproducibility studies:

- Design the study so that you can characterize intra- and inter-assay reproducibility.
- Use appropriate test samples at multiple DNA concentrations, similar to the concentrations in the procedure you recommend to users. You should include both wild-type and mutation sequences. In addition, the genotype of samples or sample panels you test should, as much as possible, reflect all the alleles that are included in the test.
- Ensure that samples used in reproducibility testing are processed from "real" samples (e.g., whole blood, buccal swabs, or other intended use matrices) at the test site and that processing mimics the procedure you plan to recommend in the test labeling.

- Include 3 or more sites with multiple operators at each site. Operators should reflect potential users of the assay, in terms of education and experience. If training will be necessary for users to perform the test once it is marketed, you should provide information on operator training. If such training is not expected to be provided for users, you should not provide additional training (other than the proposed labeling, such as the package insert) at the testing sites.

- Ensure that procedures used in the reproducibility studies are the same as the procedure that you will recommend to users in the package insert.

- Include multiple product lots, and multiple instruments (if instruments are part of the test system), to adequately test the expected performance of the system.

In the study design description in your 510(k), you should identify which factors (e.g., instrument calibration, reagent lots, and operators) were held constant and which were varied during the evaluation, and describe the computations and statistical analyses used to evaluate the data.

8. Method Comparison

You should perform method comparison studies that demonstrate that your device detects the genotypes it claims to detect, and does not detect mutations when none are present. Samples used in these studies should be patient samples derived from the intended use population, in order to show that the device will perform as claimed in a clinical setting. We recommend that you perform method comparison studies at 3 or more sites that reflect potential users of the assay, in terms of experience and education.

Because of the abundance of technologies that could be used to genotype a patient's drug metabolizing enzyme(s), assays may vary significantly in terms of methodology, instrumentation, and sample source, making direct comparison difficult. You should compare results of your device to bidirectional DNA sequence analysis.

You should describe the protocol and results of your method comparison in your 510(k). You should submit, along with your comparative sequence data, a measure of sequence quality such as a phred score or percent correct sequence calls. You can then use this information to calculate the percent correct genotype calls of your device, relative to the bidirectional sequence data. We recommend that you tabulate all results and indicate the percent correct calls for the various genotypes. We recommend that you resolve and explain all discrepant results in the 510(k); however, you should use original unresolved results for all performance calculations, in order to avoid bias. You should include failed assays (e.g., inability to correctly determine genotype within a sample, reporting of an incorrect result, instrument failure, or reagent failure) in your description of results. Any incorrect or absent genotype determinations should be considered disagreements for the purposes of reporting performance characteristics.

Clinical Validation

Prospective clinical testing to determine clinical validity may not be necessary for validation of DME genotyping systems, if there is an established scientific framework and sufficient body of evidence supporting the clinical validity and utility of your device. In this case, you should

provide the relevant peer-reviewed references. These should include multiple studies that test appropriate populations. In cases where the literature does not sufficiently support your indications for use, you should conduct studies (usually prospective studies) to support claims for your device. In either case, you should demonstrate the association between the drug metabolizing enzyme genotype and the drug metabolic profile based on clinical testing (such as enzymatic rate studies) of study subjects.

Study Samples

While prospective samples are preferred, well-characterized samples from banks can be used in your method comparison study, if clinical utility and validity are already established in the literature. You should use clinical samples from all matrices you claim in your intended use to demonstrate that correct results can be obtained from clinical material. You should fully describe selection (inclusion/exclusion) criteria and characterize any relevant features of the samples (whether prospective, or from banks). You should also provide clear information supporting sample integrity.

Appropriate sample size depends on factors such as reproducibility, interference, and other performance characteristics of the test. We recommend that you provide a statistical justification to support your study sample size.

We recommend that you evaluate test samples that encompass all genotypes in the test system. For rare mutant alleles, genomic DNA samples or clone blends may also be used, but the composition of the test samples generated from these clones should resemble, as closely as possible, the protein and DNA content and concentrations of real clinical samples. If you use clones, you should test them in combination with various other alleles or genetic backgrounds, so that they reflect heterozygous, as well as homozygous samples. In cases where you cannot obtain multiple samples of a rare genotype, you should test a statistically determined number of replicates so that you can calculate a meaningful reproducibility value for that allele.

Sample collection and handling conditions

We recommend that you validate statements in your labeling about sample storage and transport by assessing sample stability and recovery over the storage times and temperatures recommended to users. For example, an appropriate study may include an analysis of aliquots stored under the conditions of time, temperature, or specified number of freeze/thaw cycles. We recommend that you state your acceptance criteria for the sample stability parameters.

9. Software

If your system includes software, you should submit software documentation detailed in accordance with the level of concern (See: *Guidance for the Content of Premarket Submissions for Software Contained in Medical Devices* (http://www.fda.gov/cdrh/ode/57.html)). You should determine the Level of Concern prior to the mitigation of hazards. In vitro diagnostic devices of this type are typically considered a moderate level of concern, because software flaws could indirectly affect the patient and potentially result in injury because of the action or inaction of a healthcare provider who does not get accurate information.

You should include the following points, as appropriate, in preparing software documentation for FDA review:

- Full description of the software design. Your software should not include utilities that are specifically designed to support uses beyond those in your intended use. You should also consider privacy and security issues in your design. Information about some of these issues may be found at the following website regarding the Health Insurance Portability and Accountability Act (HIPAA) http://aspe.os.dhhs.gov/admnsimp.

- Hazard analysis based on critical thinking about the device design and the impact of any failure of subsystem components, such as signal detection and analysis, data storage, system communications and cybersecurity in relationship to incorrect patient reports, instrument failures, and operator safety.

- Documentation of complete verification and validation (V&V) activities for the version of software that will be submitted to demonstrate substantial equivalence. You should also submit information regarding validation of the compatibility of assay software with any instrumentation software.

- If the information you include in the 510(k) is based on a version other than the release version, identification of all differences in the 510(k) and detail how these differences (including any unresolved anomalies) impact the safety and effectiveness of the device.

Below are additional references to help you develop and maintain your device under good software life cycle practices consistent with FDA regulations.

- General Principles of Software Validation; Final Guidance for Industry and FDA Staff; available on the FDA Web site at: http://www.fda.gov/cdrh/ode/510kmod.pdf.
- Guidance for Off-the-Shelf Software Use in Medical Devices; Final; available on the FDA Web site at: http://www.fda.gov/cdrh/ode/guidance/585.pdf.
- 21 CFR 820.30 Subpart C – Design Controls of the Quality System Regulation.
- ISO 14971-1; Medical devices - Risk management - Part 1: Application of risk analysis.
- AAMI SW68:2001; Medical device software - Software life cycle processes.

10. Labeling

The premarket notification should include labeling in sufficient detail to satisfy the requirements of 21 CFR 807.87(e). The following suggestions are aimed at assisting you in preparing labeling that satisfies the requirements of 21 CFR 807.87(e). Although final labeling is not required for 510(k) clearance, final labeling for in vitro diagnostic devices must comply with the requirements of 21 CFR 809.10 before an *in vitro* diagnostic device is introduced into interstate commerce.

Directions for use

You should present clear and concise instructions that delineate the technological features of the specific device and how the device is to be used. Instructions

should encourage local/institutional training programs, if available, that are designed to familiarize users with the features of the device and how to use it in a safe and effective manner.

If you do not intend to provide reagents for DNA extraction and preparation as part of the assay, you should provide specifications for reagents needed and for assessing the quality of the assay input DNA, so that the user can select appropriate reagents.

Interpretation of Results

You should clearly define any phenotype definitions (e.g., Extensive, Intermediate, Poor, or Ultrarapid Metabolizers in the case of cytochrome P4502D6). We recommend that you provide a section in your package insert to aid users in interpreting test results. The results should be consistent with current recommendations of genetics professional societies, if applicable, and should contain adequate interpretation guidelines for the use of the ordering physician. See ***Test Results*** in Section 6. Where applicable, reports should describe the polymorphisms identified by your test and the methodology and technology used for detection. You should identify representative literature references describing genotypic interpretations, or phenotypic predictions, if applicable, to enable users to access information about specific genotypes.

Expected Values

You should provide data concerning prevalence for specific alleles, including, where appropriate, allele prevalence according to ethnicity and race.

Quality Control

You should include a description of quality control recommendations in the package insert. This should include a clear explanation of what controls are to be used in the assay and the expected results for the control material.

Precautions for interpretations

You should clearly describe any assay limitations in the labeling. Most drug metabolizing enzyme genotyping systems should contain the following limitations:

- Results provided by this type of genetic test should only be used to supplement other tools for therapeutic decision-making in conjunction with routine monitoring by a physician.

- The effect that a specific DME allele has on drug metabolism may vary depending on the specific drug, even for drugs within a specific class. Effects of specific alleles on drug metabolism are well-documented for some drugs; for other drugs, they are less well-documented. Therefore, clinicians should use professional judgment when interpreting results from this type of test.

- Results from this type of assay should not be used to predict a patient's response to drugs in cases where either 1) the drug metabolizing enzyme activity of the

allele has not been determined, or 2) the drug's metabolic pathway has not been clearly established.

Performance Characteristics

You should include in the package insert all study designs and results for studies described in Sections 7 and 8 of this guidance document that would aid users in interpreting test results. For the method comparison, you should describe device performance in comparison to a gold standard method, such as bi-directional DNA sequencing. We recommend that you present results in the form of tables (e.g., n x n tables), descriptions of percent correct genotype calls relative to sequence analysis, and a list of the nature of any miscalls (e.g., correct sequence versus one predicted by device). We recommend that you present results for specific genotypes, in addition to overall results.

Survey

MEDICINES IN DEVELOPMENT FOR

Women

PRESENTED BY AMERICA'S PHARMACEUTICAL COMPANIES

女性に関わる300種を超える疾病用治療薬が開発中

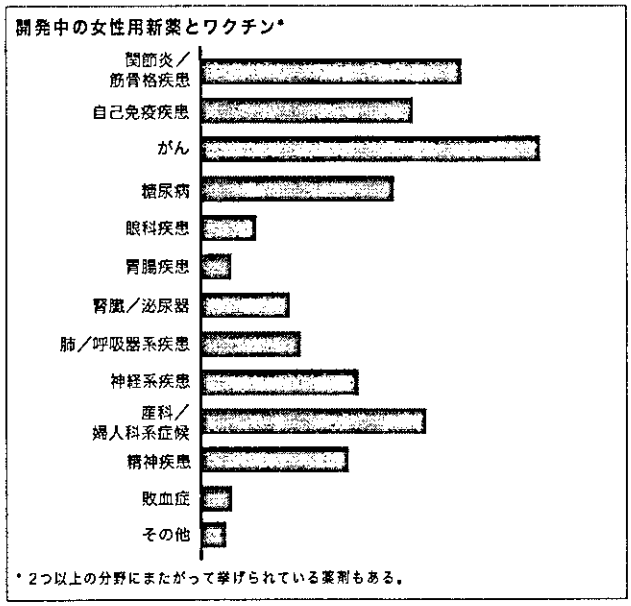
現在、米国の研究開発型製薬会社が開発に取組んでいる女性が特に罹りやすい疾病の治療薬は371種に及ぶ。

米国研究製薬工業協会（PhRMA）が行った最近の調査により、現在開発パイプライン上（臨床試験段階または米食品医薬品局（FDA）の認可待ち段階のいずれかにある）にある女性のための新薬となる医薬品には以下のものがあることが判っている。

- **がん治療薬**：71種（乳がん治療薬41種、卵巣がん治療薬34種、子宮頸がん治療薬10種）
- **関節炎／筋骨格疾患治療薬**：55種。米国人女性4,100万人が関節炎に罹っている。筋骨格疾患による米国経済の損失（直接的費用、逸失賃金、生産性の低下）は年間約1,250億ドルにのぼる。
- **産科／婦人科系症候の治療薬**：48種。これらは、毎年18歳～50歳までの女性450万人に見られる。
- **自己免疫疾患治療薬**：45種。自己免疫疾患は、米国女性の障害の要因第4位で、罹患率は男性の2.7倍。
- **糖尿病治療薬**：41種。糖尿病は特に女性や少数民族で多い。成人女性のおよそ9%が糖尿病に罹患している。そのうち3人に1人は自覚がない。
- **抗うつ剤**：23種。女性の大うつ病性障害の罹患率は男性の約2倍。
- **アルツハイマー病治療薬**：21種。女性のアルツハイマー病罹患率は男性より2～3倍高く、80歳以上の女性では50%～70%が罹患している。
- **喘息治療薬**：20種。2001年には3,100万人超の米国人が喘息に罹った。女性の罹患率は男性の1.3倍。

開発中の女性用新薬の一例を次に挙げる。

- **新型多発性硬化症治療薬**：ヒト化モノクローナル抗体で、免疫細胞の血管壁への付着や、炎症細胞の血管から組織への移動を防ぐ全く新しいタイプの薬。
- **関節リウマチ治療薬**：「トラップ」技術を用いて病状進行を防ぐ。
- **転移性乳癌新治療薬**：腫瘍を増大させる血管形成に大きな役目を果たすタンパク質の血管内皮細胞増殖因子（VEGF）に結合して阻害する。



PhRMAが別途行った調査によると、各製薬会社の研究機関は毎年50万人の女性を死に至らしめる心臓病および脳卒中の治療薬123種、女性を死に至らしめる主要ながんである肺がんの治療薬70種の開発に取組んでいることが判っている（詳細についてはP28を参照）。

フリーダ・ルイス・ホール博士は、女性用新薬の開発に取組む企業は、「女性の健康」について「(女性用新薬の課題は）単に妊娠中の健康状態や小児の健康に留まらず、より広く、深くなってきた。また、どれだけ長く生きるかではなく、どれだけ豊かに生きるか、どのようなクオリティ・オブ・ライフ（QOL）を得るかがテーマになっている。企業は、男女別の研究を行い、その類似点と相違点を考察している。これにより、現在そして将来にわたって、さらに女性をターゲットにした薬剤を提供していくことが可能となる」と、その定義を最近塗り替えている、と指摘している。

Alan F. Holmer

PhRMA理事長
アランF.ホーマー

開発中の女性用新薬

関節炎／筋骨格疾患

Product Name	Company	Indication	Development Status*
423557 (calcium antagonist)	GlaxoSmithKline *Philadelphia, PA* *Rsch. Triangle Park, NC*	osteoporosis	Phase I (888) 825-5249
462795 (cathepsin K inhibitor)	GlaxoSmithKline *Philadelphia, PA* *Rsch. Triangle Park, NC*	osteoarthritis, osteoporosis	Phase I (888) 825-5249
681323 (p38 kinase inhibitor)	GlaxoSmithKline *Philadelphia, PA* *Rsch. Triangle Park, NC*	rheumatoid arthritis	Phase I (888) 825-5249
ABT-963	Abbott Laboratories *Abbott Park, IL*	arthritis	Phase I (847) 936-1189
AGIX-4207	AtheroGenics *Alpharetta, GA*	rheumatoid arthritis	Phase II (678) 336-2500
ALX 1-11 recombinant human parathyroid hormone (rhPTH)	NPS Pharmaceuticals *Salt Lake City, UT*	osteoporosis	Phase III (801) 583-4939
AnergiX.RA™	Corixa *Seattle, WA*	rheumatoid arthritis	Phase I/II (206) 754-5711
Arcoxia™ etoricoxib	Merck *Whitehouse Station, NJ*	osteoarthritis, rheumatoid arthritis (see also obstetric/gynecologic)	application submitted (800) 672-6372
arzoxifene	Eli Lilly *Indianapolis, IN*	osteoporosis	Phase III (800) 545-5979
AZD3582	AstraZeneca *Wilmington, DE*	osteoarthritis	Phase II www.astrazeneca.com
AZD8309	AstraZeneca *Wilmington, DE*	rheumatoid arthritis	Phase I www.astrazeneca.com
AZD9056	AstraZeneca *Wilmington, DE*	rheumatoid arthritis	Phase I www.astrazeneca.com
bazedoxifene	Ligand Pharmaceuticals *San Diego, CA* Wyeth Pharmaceuticals *Collegeville, PA*	prevention and treatment of postmenopausal osteoporosis	Phase III (610) 902-1200
		prevention and treatment of postmenopausal osteoporosis in combination with conjugated estrogens	Phase III (610) 902-1200
Boniva™ ibandronate	GlaxoSmithKline *Philadelphia, PA* *Rsch. Triangle Park, NC* Roche *Nutley, NJ*	treatment and prevention of post-menopausal osteoporosis (monthly oral dosing)	Phase III (888) 825-5249 (973) 235-5000
		treatment and prevention of post-menopausal osteoporosis (intermittent i.v. dosing)	Phase III (888) 825-5249 (973) 235-5000

* For more information about a specific medicine in this report, please call the telephone number listed.

関節炎／筋骨格疾患

Product Name	Company	Indication	Development Status*
CDP-870	Pfizer *New York, NY*	rheumatoid arthritis	Phase III (860) 732-6058
clenoliximab/ IDEC-151	Biogen Idec *Cambridge, MA* *San Diego, CA*	rheumatoid arthritis	Phase II (617) 679-2000
COX 189	Novartis Pharmaceuticals *East Hanover, NJ*	osteoarthritis, rheumatoid arthritis	application submitted (888) NOW-NOVA
CP-533,536	Pfizer *New York, NY*	osteoporosis	Phase I (860) 732-6058
CTLA4Ig	Bristol-Myers Squibb *Princeton, NJ*	rheumatoid arthritis	Phase III (212) 546-4000
eculizumab	Alexion Pharmaceuticals *Cheshire, CT*	rheumatoid arthritis (see also kidney/urologic)	Phase II (203) 272-2596
Fortical® nasal calcitonin	Unigene Laboratories *Fairfield, NJ* Upsher-Smith Laboratories *Maple Grove, MN*	osteoporosis	application submitted (973) 882-0860
Hectorol™ doxercalciferol	Bone Care International *Madison, WI*	osteoporosis (see also autoimmune)	Phase II (608) 662-7800
Humira® adalimumab	Abbott Laboratories *Abbott Park, IL*	early rheumatoid arthritis, juvenile rheumatoid arthritis	Phase III
IL-1 trap	Regeneron Pharmaceuticals *Tarrytown, NY*	rheumatoid arthritis	Phase II
ISIS 104838	Isis Pharmaceuticals *Carlsbad, CA*	rheumatoid arthritis (see also autoimmune)	Phase II (760) 931-9200
lasofoxifene	Ligand Pharmaceuticals *San Diego, CA* Pfizer *New York, NY*	osteoporosis (see also cancer)	Phase III (860) 732-6058
Menostar™ ultra-low dose estradiol transdermal system	Berlex Laboratories *Montville, NJ* 3M Pharmaceuticals *St. Paul, MN*	osteoporosis	application submitted (973) 487-2461
Miacalcin **Nasal Spray** calcitonin-salmon	Novartis Pharmaceuticals *East Hanover, NJ*	osteoporosis	application submitted (888) NOW-NOVA
MM-093 (recombinant human alpha-fetoprotein)	Merrimack Pharmaceuticals *Cambridge, MA*	rheumatoid arthritis	Phase I
NGD-2001	Neurogen *Branford, CT*	rheumatoid arthritis (see also lung/respiratory)	Phase II (203) 488-8201
NOX-1094	Medinox *San Diego, CA*	arthritis	Phase I
oral salmon calcitonin	Emisphere Technologies *Tarrytown, NY*	post-menopausal osteoporosis	Phase I (914) 785-4747

関節炎／筋骨格疾患

Product Name	Company	Indication	Development Status*
Oratonin™ oral calcitonin	Nobex *Rsch. Triangle Park, NC* Synerobex *Rsch. Triangle Park, NC*	osteoporosis	Phase I
osteoprotegerin (OPG)	Amgen *Thousand Oaks, CA*	osteoporosis	Phase II (800) 772-6436
parecoxib	Pfizer *New York, NY*	arthritis	Phase III (860) 732-6058
Paxceed™ micellar paclitaxel for injection	Angiotech Pharmaceuticals *Vancouver, British Columbia*	rheumatoid arthritis (see also autoimmune)	Phase II (604) 221-7676
PEG-sTNF-R1	Amgen *Thousand Oaks, CA*	rheumatoid arthritis	Phase II (800) 772-6436
Pennsaid® Topical Solution topical diclofenac	Dimethaid Research *Markham, Ontario*	osteoarthritis	application submitted (905) 415-1446
pralnacasan	Aventis Pharmaceuticals *Bridgewater, NJ* Vertex Pharmaceuticals *Cambridge, MA*	osteoarthritis, rheumatoid arthritis	Phase II (617) 444-6100
PREOS recombinant human parathyroid hormone (1-84)	NPS Pharmaceuticals *Salt Lake City, UT*	osteoporosis	Phase III (801) 583-4939
Prograf tacrolimus	Fujisawa Healthcare *Deerfield, IL*	rheumatoid arthritis	Phase III (800) 888-7704
R1487 (kinase inhibitor)	Roche *Nutley, NJ*	rheumatoid arthritis	Phase I (973) 235-5000
R1569 (MRA) (humanized anti-IL-6 recombinant MAb)	Roche *Nutley, NJ*	rheumatoid arthritis	Phase II (973) 235-5000
Remicade® infliximab	Johnson & Johnson Pharmaceutical Research & Development *Raritan, NJ*	early rheumatoid arthritis juvenile rheumatoid arthritis (see also autoimmune)	Phase III (800) 817-5286
rhIGF-I/rhIGFBP-3	INSMED *Richmond, VA*	osteoporosis (see also diabetes)	Phase II (804) 565-3022
r-IL-18 bp	Serono *Rockland, MA*	rheumatoid arthritis (see also autoimmune)	Phase I (800) 283-8088
Rituxan® rituximab	Biogen Idec *Cambridge, MA* *San Diego, CA* Genentech *South San Francisco, CA* Roche *Nutley, NJ*	refractory rheumatoid arthritis	Phase III (617) 679-2000 (650) 225-1000 (973) 235-5000
		moderate-to-severe rheumatoid arthritis	Phase II (617) 679-2000 (650) 225-1000 (973) 235-5000

関節炎／筋骨格疾患

Product Name	Company	Indication	Development Status*
SC-080,036	Pfizer *New York, NY*	rheumatoid arthritis	Phase I (860) 732-6058
SCIO-323	Scios *Sunnyvale, CA*	rheumatoid arthritis	Phase I
SCIO-469	Scios *Sunnyvale, CA*	rheumatoid arthritis	Phase II
SERM 3471	Aventis Pharmaceuticals *Bridgewater, NJ*	post-menopausal osteoporosis	Phase II (973) 394-6000
Synvisc® hylan GF 20	Genzyme *Cambridge, MA*	osteoarthritis (for the hip)	Phase III (800) 745-4447
tibolone (OD-14)	Organon *Roseland, NJ*	osteoporosis (see also obstetric/gynecologic)	application submitted (800) 241-8812
Vitaxin™	MedImmune *Gaithersburg, MD*	rheumatoid arthritis	Phase II (301) 417-0770
zoledronic acid	Novartis Pharmaceuticals *East Hanover, NJ*	osteoporosis	Phase II (888) NOW-NOVA

自己免疫疾患

Product Name	Company	Indication	Development Status
Where to find additional autoimmune diseases—medicines in development for rheumatoid arthritis are listed under Arthritis/Musculoskeletal Disorders; medicines for diabetes are listed under Diabetes.			
683699 (dual alpha4 integrin antagonist)	GlaxoSmithKline *Philadelphia, PA* *Rsch. Triangle Park, NC*	multiple sclerosis	Phase I (888) 825-5249
Amevive® alefacept	Biogen Idec *Cambridge, MA* *San Diego, CA*	scleroderma	Phase I (617) 679-2000
Ampligen®	HEMISPHERx Biopharma *Philadelphia, PA*	chronic fatigue syndrome	Phase III
Antegren® natalizumab	Biogen Idec *Cambridge, MA* *San Diego, CA*	multiple sclerosis (relapsing forms)	Phase III (617) 679-2000
	Elan Pharmaceuticals *South San Francisco, CA*	multiple sclerosis (relapsing forms) in combination with **Avonex®**	Phase III (617) 679-2000
anti-CD154 MAb	Biogen Idec *Cambridge, MA* *San Diego, CA*	immune thrombocytopenic purpura	Phase I (617) 679-2000
anti-TGF beta **(Orphan Drug)**	Genzyme *Cambridge, MA*	diffuse scleroderma	Phase I/II (800) 745-4447
Aslera™ prasferone **(Orphan Drug)**	Genelabs Technologies *Redwood City, CA*	systemic lupus erythematosus	Phase III completed (650) 369-9500
ASM981	Novartis Pharmaceuticals *East Hanover, NJ*	psoriasis	Phase II (800) NOW-NOVA

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Product Name	Company	Indication	Development Status
Avandia® (PPAR gamma agonist)	GlaxoSmithKline *Philadelphia, PA* *Rsch. Triangle Park, NC*	psoriasis	Phase III (888) 825-5249
Avonex® interferon beta-1a **(Orphan Drug)**	Biogen Idec *Cambridge, MA* *San Diego, CA*	secondary progressive multiple sclerosis	Phase III (617) 679-2000
		combination trials	Phase II (617) 679-2000
AZD4750	AstraZeneca *Wilmington, DE*	multiple sclerosis	Phase I www.astrazeneca.com
BIRB 796	Boehringer Ingelheim Pharmaceuticals *Ridgefield, CT*	psoriasis	Phase II (203) 798-9988
Copaxone® gatiramer acetate	TEVA Neuroscience *North Wales, PA*	primary-progressive multiple sclerosis	Phase III (215) 591-3000
cyclobenzaprine (very low dose)	Vela Pharmaceuticals *Lawrenceville, NJ*	fibromyalgia syndrome	Phase II completed
Enbrel® etanercept	Amgen *Thousand Oaks, CA* Wyeth Pharmaceuticals *Collegeville, PA*	psoriasis	application submitted (800) 772-6426 (610) 902-1200
fampridine-SR	Acorda Therapeutics *Hawthorne, NY*	multiple sclerosis	Phase II (914) 347-4300
Hectorol™ doxercalciferol	Bone Care International *Madison, WI*	psoriasis (see also arthritis/musculoskeletal)	Phase I (608) 662-7800
HuMax-CD4	Genmab *Princeton, NJ* *Copenhagen, Denmark*	psoriasis	Phase II (609) 430-2481
IDEC-131	Biogen Idec *Cambridge, MA* *San Diego, CA*	immune thrombocytopenic purpura, psoriasis	Phase II (617) 679-2000
IFNAR-2	Serono *Rockland, MA*	multiple sclerosis	Phase I (800) 283-8088
intranasal interferon beta	Nastech Pharmaceutical *Bothell, WA*	multiple sclerosis	Phase I (425) 908-3600
IR-208	Immune Response *Carlsbad, CA*	multiple sclerosis	Phase I/II (760) 431-7080
ISIS 104838	Isis Pharmaceuticals *Carlsbad, CA*	psoriasis (see also arthritis/musculoskeletal)	Phase II (760) 931-9200
ISIS 107248 (ATL1102)	Antisense Therapeutics *Melbourne, Australia* Isis Pharmaceuticals *Carlsbad, CA*	multiple sclerosis	Phase I (760) 931-9200
J695	Abbott Laboratories *Abbott Park, IL*	autoimmune diseases	Phase II (847) 935-9545

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Product Name	Company	Indication	Development Status
LTBR MAb (lymphotoxin beta soluble receptor)	Biogen Idec *Cambridge, MA* *San Diego, CA*	autoimmune diseases	Phase I (617) 679-2000
MDX-018	Medarex *Princeton, NJ*	autoimmune diseases	Phase I (609) 430-2880
milnacipran	Cypress Biosciences *San Diego, CA*	fibromyalgia syndrome	Phase III (858) 452-2323
NBI-5788	Neurocrine Biosciences *San Diego, CA*	multiple sclerosis	Phase II (858) 658-7600
Neurodex	Avanir Pharmaceuticals *San Diego, CA*	pathological laughing or crying associated with multiple sclerosis (emotional lability or pseudobulbar effect)	Phase III (858) 622-5200
onercept (r-TBP-1)	Serono *Rockland, MA*	psoriasis	Phase II (800) 283-8088
ONTAK® denileukin diftitox	Ligand Pharmaceuticals *San Diego, CA*	severe psoriasis	Phase II (858) 550-7500
oral fumarate	Biogen Idec *Cambridge, MA* *San Diego, CA*	psoriasis	Phase II (617) 679-2000
		multiple sclerosis (relapsing forms)	Phase I (617) 679-2000
Paxceed™ micellar paclitaxel for injection	Angiotech Pharmaceuticals *Vancouver, British Columbia*	psoriasis (see also arthritis/musculoskeletal)	Phase I (604) 221-7676
PsorBan® (CGC1072)	CellGate *Sunnyvale, CA*	psoriasis	Phase II
PVAC™	Corixa *Seattle, WA*	psoriasis	Phase II (206) 754-5711
		scleroderma	Phase I (206) 754-5711
Remicade® infliximab	Johnson & Johnson Pharmaceutical Research & Development *Raritan, NJ*	psoriasis (see also arthritis/musculoskeletal)	Phase II/III (800) 817-5286
r-IL-18 bp	Serono *Rockland, MA*	psoriasis (see also arthritis/musculoskeletal)	Phase I (800) 283-8088
Riquent abetimus sodium **(Orphan Drug)**	La Jolla Pharmaceutical *San Diego, CA*	systemic lupus erythematosus	Phase III (888) 305-8787
SR 57746	Sanofi-Synthelabo *New York, NY*	multiple sclerosis (see also neurologic)	Phase II (212) 551-4000
TACI-Ig	ZymoGenetics *Seattle, WA*	autoimmune diseases	Phase I

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Product Name	Company	Indication	Development Status
Targretin® Capsules bexarotene	Ligand Pharmaceuticals *San Diego, CA*	moderate to severe psoriasis (see also cancer)	Phase II (858) 550-7500
Targretin® Gel bexarotene	Ligand Pharmaceuticals *San Diego, CA*	psoriasis	Phase II (858) 550-7500
tazarotene (oral)	Allergan *Irvine, CA*	moderate to very severe psoriasis	application submitted (800) 433-8871
Veldona® interferon-alpha lozenges	Amarillo Biosciences *Amarillo, TX*	Sjogren's syndrome	Phase III (806) 376-1741
		fibromyalgia syndrome	Phase II (806) 376-1741

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Product Name	Company	Indication	Development Status
572016 (ErbB-2 and EGFR dual kinase inhibitor)	GlaxoSmithKline *Philadelphia, PA* *Rsch. Triangle Park, NC*	breast	Phase III (888) 825-5249
Actimmune® interferon gamma-1b	Genentech *South San Francisco, CA* InterMune *Brisbane, CA*	ovarian	Phase III (650) 225-1000 (415) 466-2200
Advexin®	Introgen Therapeutics *Austin, TX*	breast	Phase II (512) 708-9310
		ovarian	Phase I (512) 708-9310
Avastin™ bevacizumab	Genentech *South San Francisco, CA*	metastatic breast	Phase III (650) 225-1000
	National Cancer Institute *Bethesda, MD* Genentech *South San Francisco, CA*	breast, cervical, ovarian	Phase I/II/III NCI TRIAL (800) 4-CANCER
BMS-247550 (epithilone B analog)	National Cancer Institute *Bethesda, MD* Bristol-Myers Squibb *Princeton, NJ*	breast, ovarian	Phase II NCI TRIAL (800) 4-CANCER
bryostatin 1	National Cancer Institute *Bethesda, MD*	ovarian	Phase II NCI TRIAL (800) 4-CANCER
CAI	National Cancer Institute *Bethesda, MD*	ovarian	Phase II NCI TRIAL (800) 4-CANCER
CCX2	Oncosense *Cambridge, UK*	ovarian	in clinical trials

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Product Name	Company	Indication	Development Status
CEA-CIDE™	Immunomedics *Morris Plains, NJ*	ovarian	Phase I (973) 605-8200
CEA-SCAN®	Immunomedics *Morris Plains, NJ*	breast	Phase III (973) 605-8200
CEA-TRICOM vaccine	National Cancer Institute *Bethesda, MD* Therion Biologics *Cambridge, MA*	breast	Phase I/II NCI TRIAL (800) 4-CANCER
dendritic/cancer cell fusion (chemical fusion process)	Genzyme *Cambridge, MA*	breast	Phase I/II completed (800) 745-4447
DOXIL® doxorubicin HCl liposome injection	Johnson & Johnson Pharmaceutical Research & Development *Raritan, NJ*	breast	Phase III (800) 817-5286
E1A lipid complex	Targeted Genetics *Seattle, WA*	ovarian	Phase I (206) 623-7612
E7070	Eisai *Teaneck, NJ*	breast	Phase II (888) 274-2378
EF-5	National Cancer Institute *Bethesda, MD*	ovarian	Phase I NCI TRIAL (800) 4-CANCER
Enhanzyn (adjuvant)	Corixa *Seattle, WA*	breast	Phase III (206) 616-1823
ERA923	Ligand Pharmaceuticals *San Diego, CA*	breast	Phase II (858) 550-7500
Evista® raloxifene	Eli Lilly *Indianapolis, IN*	breast cancer prevention	Phase III (800) 545-5979
Faslodex® fulvestrant	AstraZeneca *Wilmington, DE*	first-line advanced breast	Phase III www.astrazeneca.com
		second-line advanced breast	Phase III www.astrazeneca.com
Femara letrozole	Novartis Pharmaceuticals *East Hanover, NJ*	early stage breast	in clinical trials (888) NOW-NOVA
fenretinide	National Cancer Institute *Bethesda, MD*	ovarian	Phase II NCI TRIAL (800) 4-CANCER
G-3139	Genta *Berkeley Heights, NJ*	breast	Phase I/II completed (908) 286-9800
GEM® 231	Hybridon *Cambridge, MA*	breast, cervical, endometrial, ovarian	Phase I/II (617) 679-5593
Gemzar® gemcitabine	Eli Lilly *Indianapolis, IN*	ovarian	application submitted (800) 545-5979

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Product Name	Company	Indication	Development Status
Gleevec™ imatinib mesylate	National Cancer Institute *Bethesda, MD* Novartis Pharmaceuticals *East Hanover, NJ*	breast, ovarian	Phase II NCI TRIAL (800) 4-CANCER
H11 scFv	Viventia Biotech *Toronto, Ontario*	breast	Phase I/II (416) 291-1277
Herceptin® trastuzumab	National Cancer Institute *Bethesda, MD* Genentech *South San Francisco, CA*	breast	Phase I/II/III NCI TRIAL (800) 4-CANCER
HPV16 E6 and E7 peptide vaccine	National Cancer Institute *Bethesda, MD*	cervical	Phase I NCI TRIAL (800) 4-CANCER
human papilloma- virus (HPV) vaccine	Merck *Whitehouse Station, NJ*	cervical cancer prevention (see also obstetric/gynecologic)	Phase III (800) 672-6372
Hycamtin™ topotecan	GlaxoSmithKline *Philadelphia, PA* *Rsch. Triangle Park, NC*	ovarian (first-line therapy)	Phase III (888) 825-5249
INGN 241 (mda7)	Introgen Therapeutics *Austin, TX*	breast	Phase II (512) 708-9310
interleukin-12	National Cancer Institute *Bethesda, MD*	ovarian	Phase II NCI TRIAL (800) 4-CANCER
Iressa® gefitinib	AstraZeneca *Wilmington, DE*	breast	Phase II www.astrazeneca.com
irofulven (MGI-114) **(Orphan Drug)**	MGI Pharma *Bloomington, MN*	ovarian	Phase I/II (800) 562-5580
lasofoxifere	Ligand Pharmaceuticals *San Diego, CA* Pfizer *New York, NY*	breast cancer prevention (see also arthritis/musculoskeletal)	Phase III (860) 732-6058
Lutrin® motexafin lutetium (photodynamic cancer therapy)	National Cancer Institute *Bethesda, MD* Pharmacyclics *Sunnyvale, CA*	cervical	Phase I NCI TRIAL (800) 4-CANCER
MAb antibody 3A1	National Cancer Institute *Bethesda, MD*	breast	Phase I/II NCI TRIAL (800) 4-CANCER
MDX-010	Medarex *Princeton, NJ*	breast	Phase II (908) 479-2400
MEDI-517 HPV vaccine	MedImmune *Gaithersburg, MD*	cervical	Phase II completed (301) 417-0770

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Product Name	Company	Indication	Development Status
Multikine leukocyte interleukin injection	CEL-SCI *Vienna, VA*	cervical cancer (see also obstetric/gynecologic)	Phase I (703) 506-9460
Myocet™ liposomal doxorubicin	Elan Pharmaceuticals *South San Francisco, CA*	metastatic breast	Phase III (800) 537-8899
Navelbine® vinorelbine tartrate	GlaxoSmithKline *Philadelphia, PA* *Rsch. Triangle Park, NC*	breast	Phase III (888) 825-5249
Omnitarg™ pertuzumab	Genentech *South San Francisco, CA*	ovarian	Phase II (650) 225-1000
OSI-211	OSI Pharmaceuticals *Melville, NY*	ovarian	Phase II (631) 962-2000
OvaRex® oregovomab **(Orphan Drug)**	United Therapeutics *Rsch.Triangle Park, NC*	ovarian	Phase II (919) 485-8350
oxaliplatin	Sanofi-Synthelabo *New York, NY*	ovarian	Phase II (212) 551-4000
P53 and RAS vaccine	National Cancer Institute *Bethesda, MD*	breast, ovarian	Phase I/II N C I Trial (800) 4-CANCER
PD-183805	Pfizer *New York, NY*	breast	Phase II (860) 732-6058
perifosine	National Cancer Institute *Bethesda, MD* AOI Pharmaceuticals *Stamford, CT*	breast	Phase II N C I Trial (800) 4-CANCER
peripheral blood lymphocytes transduced with a gene encoding a chimeric T-cell receptor	National Cancer Institute *Bethesda, MD*	ovarian	Phase I N C I Trial (800) 4-CANCER
PV701 (Newcastle disease virus)	National Cancer Institute *Bethesda, MD* WellStat Biologics *Gaithersburg, MD*	ovarian	Phase I N C I Trial (800) 4-CANCER
R1549 (MAb)	Roche *Nutley, NJ*	ovarian	Phase III (973) 235-5000
R1550 (MAb)	Roche *Nutley, NJ*	breast	Phase I (973) 235-5000
recombinant breast cancer therapeutic vaccine (Her 2 neu)	GlaxoSmithKline *Philadelphia, PA* *Rsch. Triangle Park, NC*	treatment of breast cancer	Phase I (888) 825-5249
RSR-13 (efaproxiral)	Allos Therapeutics *Denver, CO*	cervical	Phase I (303) 426-6262

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Product Name	Company	Indication	Development Status
rubitecan (RFS 2000, 9-nitro-camptothecin)	SuperGen *Dublin, CA*	breast, cervical, ovarian	Phase II (925) 560-0100
SGN-15	Seattle Genetics *Bothell, WA*	breast	Phase II completed (425) 527-4000
		ovarian	Phase I completed (425) 527-4000
suramin	National Cancer Institute *Bethesda, MD*	breast	Phase I/II NCI TRIAL (800) 4-CANCER
Tarceva™ erlotinib HCl	National Cancer Institute *Bethesda, MD* Genentech *South San Francisco, CA* Roche *Nutley, NJ* OSI Pharmaceuticals *Uniondale, NY*	breast, cervical, endometrial, ovarian	Phase II NCI TRIAL (800) 4-CANCER
Targretin® Capsules bexarotene	Ligand Pharmaceuticals *San Diego, CA*	advanced breast (see also autoimmune)	Phase II (858) 550-7500
Taxotere®	Aventis Pharmaceuticals *Bridgewater, NJ*	breast (adjuvant)	Phase III (973) 394-6000
Telcyta™ TLK286	Telik *Palo Alto, CA*	ovarian	Phase III (866) 485-5286
		breast	Phase II (866) 485-5286
tgDDC-E1A	Targeted Genetics *Seattle, WA*	ovarian	Phase I (206) 623-7612
Thalomid® thalidomide	National Cancer Institute *Bethesda, MD* Celgene *Warren, NJ*	ovarian	Phase II NCI TRIAL (800) 4-CANCER
Theratope	Biomira *Edmonton, Alberta*	metastatic breast	Phase III (780) 450-3761 x-500
		metastatic breast in patients receiving aromatase inhibitors	Phase II (780) 450-3761 x-500
tirapazamine	National Cancer Institute *Bethesda, MD* Sanofi-Synthelabo *New York, NY*	ovarian	Phase II NCI TRIAL (800) 4-CANCER
UCN-01	National Cancer Institute *Bethesda, MD* Kyowa Pharmaceuticals *East Hanover, NJ*	ovarian	Phase I NCI TRIAL (800) 4-CANCER
Velcade™ bortezomib	Millennium Pharmaceuticals *Cambridge, MA*	breast	Phase I (866) VELCADE