中国先锋医药控股有限公司

行业信息简报

编　　辑　　沈　亮　亮

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2017-05-11　CFDA

为进一步深化审评审批制度改革，促进药品医疗器械产业结构调整和技术创新，提高产业竞争力，满足公众临床需要，国家食品药品监督管理总局商国务院有关部门起草了《关于鼓励药品医疗器械创新加快新药医疗器械上市审评审批的相关政策》（征求意见稿），现向社会公开征求意见。建议将修改意见于 2017 年 5 月 25 日前通过电子邮件反馈至国家食品药品监督管理总局（药品化妆品注册管理司）。征求意见截止时间为 6 月 10 日。

征求意见稿中涉及法律法规修订的内容按相关程序进行。

电子邮件：yhzcszhc@cfda.gov.cn

特此公告。

附件：关于鼓励药品医疗器械创新加快新药医疗器械上市审评审批的相关政策（征求意见稿）

食品药品监管总局

2017 年 5 月 11 日

附件

关于鼓励药品医疗器械创新加快新药医疗器械上市审评审批的相关政策（征求意见稿）

一、加快临床急需药品医疗器械审评审批。对于治疗严重危及生命且尚无有效治疗手段疾病的药品医疗器械以及其他解决临床需求具有重大意义的药品医疗器械，临床试验早期、中期指标显示疗效并可预测其临床价值的，可有条件批准上市。申请人要制定风险管控计划，按要求开展确证性临床试验并完成批件中规定的研究内容。鼓励创新药物和医疗器械的研

发，对列入国家科技重大专项和国家重点研发计划支持的创新药物和医疗器械，给予优先审评审批。

二、支持罕见病治疗药物和医疗器械研发。由卫生计生部门公布罕见病目录，建立罕见病患者注册登记制度。罕见病治疗药物和医疗器械申请人可提出减免临床试验申请，加快罕见病用药医疗器械审评审批。对于国外已批准上市的罕见病治疗药物和医疗器械，可有条件批准上市，上市后在规定时间内补做相关研究。

三、严格注射剂审评审批。严格控制口服制剂改注射剂，凡口服制剂能够满足临床需求的，不批准注射制剂上市；严格控制肌肉注射制剂改静脉注射制剂，凡肌肉注射制剂能够满足临床需求的，不批准静脉注射制剂上市。大容量注射剂、小容量注射剂以及注射用无菌粉针之间互改剂型的申请，无明显临床优势的不予批准。

四、调整药用原辅料及包装材料管理模式。制定药用原辅料和包装材料备案管理办法，建立药用原辅料和包装材料备案信息平台，相关企业按要求提交备案资料并对备案信息的真实性负责。药品审评机构对在信息平台备案的药用原辅料和包装材料，与药品注册申请一并审评审批。药品生产企业对所选择的药用原辅料和包装材料的质量负责。

五、完善药品医疗器械审评制度。形成审评为主导、检查检验为支撑的技术审评体系。建立以临床医学专业人员为主，药学、药理毒理学、统计学等专业人员组成的药品审评团队负责新药审评；建立由临床医学、临床诊断、机械、电子、材料、生物医学工程等专业人员组成的医疗器械审评团队负责创新类医疗器械审评。建立项目管理人制度，负责申请人与审评员会议沟通组织工作，禁止审评人员私下与申请人沟通。建立项目审评过程中审评员与申请人会议沟通制度，Ⅰ期临床试验申报前、Ⅱ临床试验结束后Ⅲ期临床试验开始前和Ⅲ期临床试验结束后申报生产上市前三个重要节点，必须召开申请人与审评员会议进行充分讨论交流。审评期间，可以应申请人请求安排会议交流。建立专家咨询委员会制度，重大技术性争议问题由专家咨询委员会公开论证，听取申请人、审评员双方意见后提出意见，供决策参考。审评机构的审评结论全部向社会公开（涉及企业生产工艺及参数的商业秘密除外），接受社会监督。统一二类医疗器械审评技术标准，创造条件逐步实现国家统一审评。

六、支持新药临床应用。鼓励医疗机构优先采购和使用疗效明确、价格合理的新药。研究完善医疗保险药品目录动态调整机制，探索建立医疗保险药品支付标准谈判制度，支持创新药按规定纳入基本医疗保险支付范围。各地可根据疾病防治需要，组织以省（自治区、直辖市）为单位的集中采购。

七、支持中药传承和创新。贯彻落实《中华人民共和国中医药法》的有关规定，妥善处理保持中药疗效优势与现代药品开发要求的关系，妥善处理传统用药模式与现代用药需求的关系，建立完善符合中药特点的注册管理制度和技术评价体系。创新类中药，按照“新疗效”标准审评审批；改良型中药新药，应能体现临床应用优势；经典名方类中药，按照简化标准审评审批；天然药物，按照现代医学标准审评审批。开展中药上市价值评估及资源评估，引导以临床价值为导向的中药新药研发，促进中药资源可持续利用。加强中药质量控制，提高中药临床研究能力。鼓励运用现代科学技术研究开发传统中成药，支持以中药传统剂型为基础研制中药新药，促进中药产业健康发展。

八、建立基于专利强制许可的优先审评审批制度。根据《中华人民共和国专利法》相关规定，为维护公共健康、在公共安全受到重大威胁情况下，申请人可向知识产权部门提出强制许可申请，知识产权部门决定实施药品专利强制许可的，药品审评机构对获得强制许可的注册申请优先审评审批。公共安全受到重大威胁的情形和启动强制许可的程序，由卫生计生部门具体规定。

二、总局关于征求《关于鼓励药品医疗器械创新改革临床试验管理的相关政策》（征求意见稿）意见的公告（2017 年第 53 号）

2017-05-11　CFDA

为进一步深化审评审批制度改革，促进药品医疗器械产业结构调整和技术创新，提高产业竞争力，满足公众临床需要，国家食品药品监督管理总局商国务院有关部门起草了《关于鼓励药品医疗器械创新改革临床试验管理的相关政策》（征求意见稿），现向社会公开征求意见。建议将修改意见于 2017 年 5 月 25 日前通过电子邮件反馈至国家食品药品监督管理总局（药品化妆品注册管理司）。征求意见截止时间为 2017 年 6 月 10 日。

征求意见稿中涉及法律法规修订的内容按相关程序进行。

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附件：关于鼓励药品医疗器械创新改革临床试验管理的相关政策（征求意见稿）

食品药品监管总局

2017 年 5 月 11 日

附件

关于鼓励药品医疗器械创新改革临床试验管理的相关政策（征求意见稿）

一、临床试验机构资格认定改为备案管理。取消临床试验机构的资格认定。具备临床试验条件的医疗机构在食品药品监管部门指定网站登记备案后，均可接受申请人委托开展临床试验。鼓励社会资本投资设立临床试验机构，提供临床试验专业服务。临床试验主要研究者须具有高级职称，参加过 3 个以上临床试验。临床试验申请人可聘请第三方对临床试验机构是否具备条件进行评估认证。临床试验机构实施备案管理后，食品药品监管部门要加强对临床试验项目进行现场检查，检查结果向社会公开。未能通过检查的临床试验项目，相关数据将

不被食品药品监管部门接受。临床试验机构管理规定由食品药品监管部门会同卫生计生部门制定。

二、支持研究者和临床试验机构开展临床试验。支持医疗机构、医学研究机构、医药高等院校参与临床试验，将临床试验的条件与能力纳入医疗机构等级评审、临床重点学科认定的重要指标。鼓励三级甲等医疗机构、省属及以上高等本科医学院校的附属医院承接临床试验工作。对开展药物临床试验的医疗机构建立单独评价考核体系，仅用于开展临床试验的病床不计入医疗机构总病床，不规定病床效益、周转率、使用率等考评指标。鼓励医疗机构设立专职临床试验部门，配备职业化的临床试验人员。鼓励临床医生参与药品医疗器械技术创新活动。允许境外企业和科研机构在中国开展药物Ⅰ期临床试验。开展临床试验的医务人员在职务提升、职称晋升等方面与临床医生一视同仁。结合完善单位绩效工资分配激励机制，保障临床试验研究者收入水平。

三、完善伦理委员会机制。临床试验需符合伦理道德标准，保证受试者在自愿参与之前被告知足够的试验信息，理解并签署《知情同意书》，确保受试者的安全、健康和权益受到保护。开展临床试验的医疗机构要成立伦理委员会，负责审查临床试验方案并作出批准、要求修改或不批准的决定，负责对临床试验进行定期审查和实时监督并接受监管部门的检查，负责本机构研究者资质的审核和监督。各地区可根据需要设立区域性伦理委员会，负责审查、监督医疗机构承担的临床试验项目和监督研究者的资质，负责审理研究者和申请人的上诉，负责区域内医疗机构伦理委员会的工作指导。卫生计生部门、中医药管理部门和食品药品监管部门要加强对伦理委员会工作的管理指导和业务监督。

四、提高伦理审查效率。申请人在向审评机构提出临床试验申请前，应先将临床试验方案交由伦理委员会审查批准。在中国境内开展多中心临床试验的，经组长单位伦理审查后，其他成员单位伦理委员会可认可组长单位的审查结论，不再重复审查。国家医学临床研究中心及获得国家科技重大专项和国家重点研发计划支持的临床试验机构，应整合资源建立统一的伦理审查平台，逐步推进伦理审查互认。

五、优化临床试验审查程序。建立和完善申请人与审评机构的沟通交流机制。开展Ⅰ期和III期药物临床试验前，须经申请人与审评机构会议沟通后正式申请和受理。开展需审批的医疗

器械临床试验前，须经申请人与审评机构会议沟通后正式申请和受理。审评机构自受理之日起 60 个工作日后，没有给出否定或质疑的审查意见即视为同意，申请人可按照递交的方案开展临床试验。临床试验期间，发生临床试验方案变更、重大药学变更或非临床研究安全性问题的，申请人应及时将变更情况报送审评机构。发现存在安全性及其他风险的，申请人应及时修改临床试验方案、暂停或终止临床试验。审评机构要加强对临床试验全过程的审查、监督，组织对正在开展的临床试验进行现场核查，审评过程中可以组织对临床试验数据进行有因检查。

六、接受境外临床试验数据。申请人在境外取得的临床试验数据，符合中国药品医疗器械注册相关要求的，经现场检查后可用于在中国申报注册申请。境外企业在中国进行的国际多中心药物临床试验，符合中国药品注册相关要求的，完成国际多中心临床试验后可以直接提出上市申请。在中国首次申请上市的药品医疗器械，申请人应提供不存在种族差异的临床试验数据。申请人在欧洲药品管理局、美国和日本获准上市仿制药的生物等效性试验数据，符合中国药品注册相关要求的，经现场检查后可用于在中国申报仿制药注册。申请人在境外获准上市的医疗器械，除需进行临床试验审批的第三类医疗器械外，在境外获准上市时提交的临床试验数据，可作为临床试验资料用于在中国申报医疗器械注册。

七、支持拓展性临床试验。对于正在开展临床试验的用于治疗严重危及生命且尚无有效治疗手段疾病的药物医疗器械，经临床试验初步观察可能获益，且符合伦理要求的，经知情同意后可用于其他患者，其安全性数据可用于支持审评审批。拓展使用的试验药物，仅限在开展Ⅱ、Ⅲ期临床试验的机构使用，使用人数不得超过临床试验规定的受试者数量。

三、介入人工生物心脏瓣膜产品获批上市

2017-05-16 CFDA

近日，国家食品药品监督管理总局经审查，批准了苏州杰成医疗科技有限公司生产的创新产品“介入人工生物心脏瓣膜”的注册。

该产品由自膨胀介入瓣膜、经心尖介入器、瓣膜装载件组成，适用于经心脏团队结合评分系统评估后认为不适合进行外科手术的自体主动脉瓣病变患者，包括主动脉瓣狭窄患者、主动脉瓣关闭不全患者。

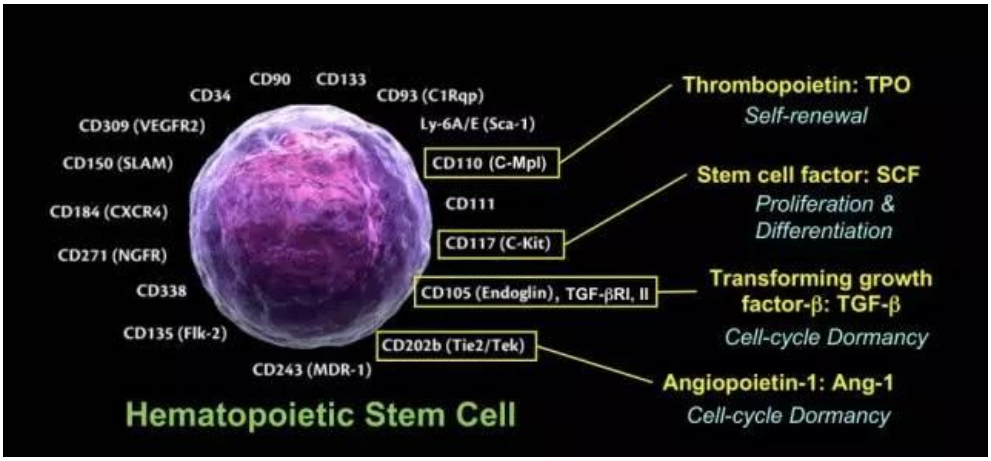
该产品在无需心脏快速起搏的情况下自动定位植入位点，用于主动脉瓣关闭不全患者的治疗属于国内首创。该产品的上市将为患有主动脉瓣狭窄患者和主动脉瓣关闭不全患者带来了显著的临床获益。

食品药品监督管理部门将加强该产品上市后监管，保护患者用械安全。

四、《自然》：20 年！科学家首次造出功能性造血干细胞样细胞

2017-05-19　环球医讯

经过 20 年的努力，科学家们终于成功地将成熟细胞转化为了具有功能性的造血干细胞样细胞！这意味着干细胞领域再次取得了不小的突破。



携带各种表面标记的造血干细胞

今天，有两篇新的研究同时刊登在了《自然》杂志上。经过 20 年的努力，科学家们终于成功地将成熟细胞转化为了具有功能性的造血干细胞样细胞!这意味着干细胞领域再次取得了不小的突破。

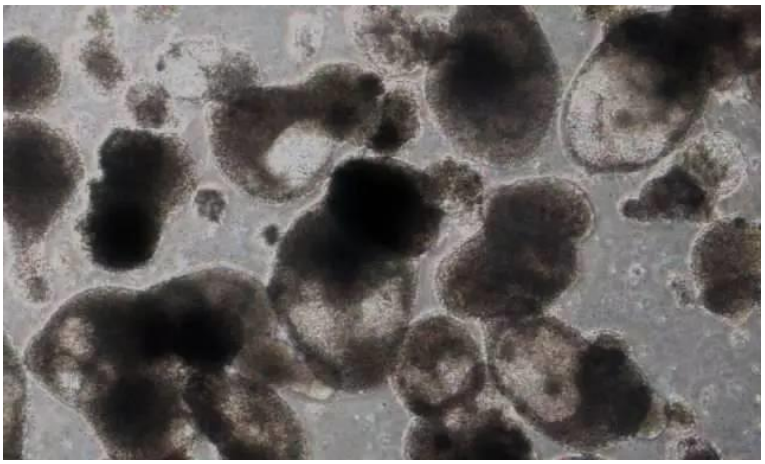
造血干细胞是在胚胎发育期间产生的，正常的造血干细胞具有长期自我更新的能力和分化成各类成熟血细胞的潜能。自从 1998 年，人的胚胎干细胞（ES）被成功分离出来，科学家们就一直在尝试着用它们去“制造”造血干细胞，但是一直没能获得成功。

因此，这次的成功对于干细胞领域无疑是一个很大的鼓励。《自然》杂志也为这两个研究撰写了评论文章，指出这些成果为白血病和其他需要骨髓移植的，但又找不到合适供体的血液疾病患者带来了希望。两项研究分别由波士顿儿童医院干细胞计划实验室的负责人 George Daley 教授和 Weill Cornell 医学院的 Shahin Rafii 教授领导。

George Daley 教授（左）和 Shahin Rafii 教授（右）

过去，Daley 教授的团队尝试过直接使用人的诱导性多能干细胞（hiPSCs）直接分化出造血干细胞，但是没有成功。于是这次他们选择了“绕路”，首先通过化学信号诱导，将人的多能干细胞（hPSCs，包括 ES 和 hiPSCs）分化成了生血内皮细胞（hemogenic endothelium cells），它具有转化为造血干细胞的能力。

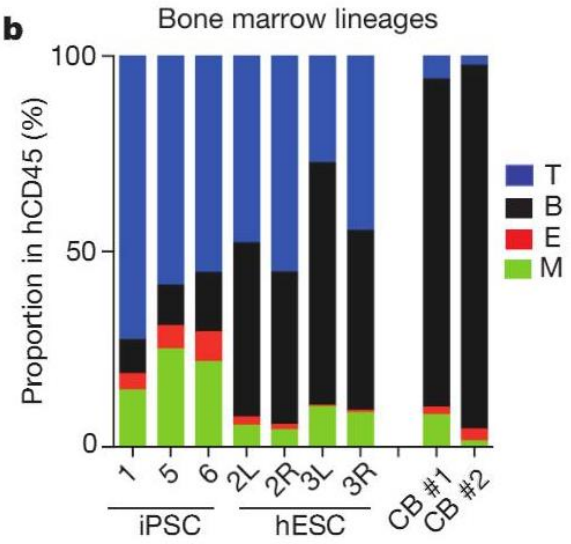
在造血细胞因子的“刺激”下，研究人员观察到了生血内皮细胞向造血干细胞转化状态的细胞（EHT），但是当他们尝试将 EHT 移植给小鼠的时候却失败了，移植的细胞并不能“造血”。



研究人员诱导出的包含 EHT 的混合态干细胞

接下来，他们“复习”了过去的干细胞分化研究，构建了一个包括 26 个转录因子的转录因子库，将它们通过病毒载体转入培养到第 3 天的 EHT 中，然后移植给有免疫缺陷的小鼠。这经过 12 周，对小鼠的骨髓和胸腺进行检查，发现了人的红细胞、骨髓细胞、B 细胞和 T 细胞。在 26 个转录因子中，研究人员最终确定了 7 个（ERG、HOXA5、HOXA9、HOXA10、

LCOR、RUNX1 和 SPI1）在 4 种细胞中都存在，他们认为，这 7 个转录因子是促使生血内皮细胞向有“造血”能力的造血干细胞样细胞转化的关键因子。对这 7 个转录因子再次进行实验，同样观察到了 4 种细胞生成。



不同细胞系中红细胞（E）、骨髓细胞（M）、B 细胞（B）和 T 细胞（T）组成比例

Daley 教授表示，他们所发现的这种细胞与天然的造血干细胞之间仍然存在一定差异，但是已经是“极其接近”的了。他们还在接下来的试验中验证了移植的细胞具有正常的自我更新能力。造血干细胞的自我更新能力非常重要，是维持机体正常造血需求的必须能力。

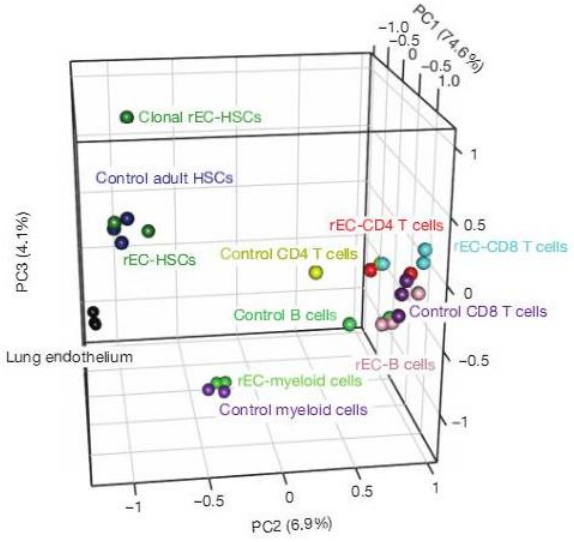
相比 Daley 教授，Rafii 教授并没有选择人源的细胞进行实验，而是直接采用了小鼠的细胞。他们从小鼠的血管内提取了内皮细胞，然后将四个转录因子（Fosb 、Gfi1、 Runx1 和 Spi1）转入细胞中，这是他们在 2014 年时候发现的，通过这 4 个转录因子可以将血管内皮细胞转化为造血干细胞样细胞。

接下来将细胞转入模拟血管环境中，这种“血管环境”充满各种细胞，能够为内皮细胞重编程和分化提供一些“信号分子”。经过 28 天的培养，就产生了具有造血干细胞功能的细胞，而这个过程中，内皮细胞不需要经历多能干细胞这一状态。

血管内皮细胞

Rafii 教授在接受《The Scientist》网站的采访时表示，他们在实验中模拟的血管环境是细胞转化成功的关键因素，“就像母亲为婴儿提供营养一样，没有母亲的支持，婴儿也不能存活，这和我们实验中的细胞是一样的。”

这些细胞也同样拥有良好的自我更新能力和分化为各类血细胞以及具有免疫活性的淋巴细胞（T 细胞和 B 细胞）的能力。体外实验获得成功后，研究人员选择了接受过放射治疗，体内大部分血细胞和免疫细胞都被杀死的小鼠，将这些诱导出的细胞移植给它们。在移植后，小鼠重新获得了造血能力，恢复到了未接受治疗前的状态，在实验室中生活了 1 年半多的时间。此外，研究人员还对小鼠进行了长达 40 周的监测，没有发现组织和血液中出现恶性病变的情况。



3D 主成分分析（PCA）图，显示诱导出的造血干细胞样细胞（rEC-HSCs）及分化出的血细胞和淋巴细胞的多维度聚集

对于这两个“殊途同归”的研究，Rafii 教授表示，他“绕过”了多能干细胞阶段，他的方法更像是一个“直达航班”，而 Daley 教授的方法则需要“中途转机”。而 Daley 则认为，他的方法效率更高，在致癌和其他病变上的可能性更小。此外，加州 Scripps 研究所的研究员 Janne Loring 表示，多能干细胞容易获得，而 Rafii 教授的方法中需要的血管内皮细胞相对难获取和存活，这会增加实际应用中的难度。

如果他们的结果能被更多的实验室所复制，那么也许还会有其他的完善和进展，将研究推入临床的可能性也会更大。Daley 教授也提出，他们想将来能够引入基因编辑技术如 CRISPR，而不再利用病毒载体，扩大实际使用中干细胞的能力和安全性，希望有更多的患者能够受益。

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五、优于二甲双胍的一类新型口服降糖药即将面世

2017-05-19　生物谷

由阿德莱德大学领导的研究为治疗 2 型糖尿病的更安全有效的药物的产生以及减少副作用和注射胰岛素的需要铺平了道路。

在“药物化学杂志”和“BBA 一般受试者”杂志上发表的两项研究首次显示出新的潜在抗糖尿病药物在分子水平上如何与其靶物质相互作用。

这些新的潜在药物具有与最常规处方的抗糖尿病药二甲双胍具有完全不同的作用（二甲双胍作用于肝脏以降低葡萄糖产量），并且在降低血糖方面可能更有效。他们针对在全身脂肪组织中发现的称为 PPARγ 的蛋白受体，使其全部或部分活化，以增加对胰岛素的敏感性和改变脂肪和糖的代谢来降低血糖。

首席研究员 John Bruning 博士说：“二型糖尿病的特点是抵抗胰岛素，随后出现高血糖，导致严重的疾病，通常与饮食不良和运动不足等生活方式不良相关。”

“自 1990 年以来，澳大利亚的 2 型糖尿病患病率已经翻了三倍，估计每年的成本为 60 亿美元，因此开发安全和更有效的治疗药物变得越来越重要。

“严重糖尿病患者需要服用胰岛素，但必须注射胰岛素是有困惑的一件事，很难使胰岛素水平正常，人们非常希望脱离胰岛素注射，而是使用口服治疗药物。”

第一项研究与美国佛罗里达州的斯克里普斯研究所合作，描述了 Rebecca Frkic 的一项荣誉研究项目，其中制作了 14 种不同版本的部分激活 PPARgamma 的药物。与完全激活相比，部分激活可以减少副作用。

原始药物 INT131 目前正在美国的临床试验中进行测试，但阿德莱德大学生产的一些药物与原来的药物相比具有更高的效力，有望进一步改善 2 型糖尿病的治疗。

Bruning 博士说：“这项研究的一个主要发现是能够显示哪些区域的药物与 PPARγ受体相互作用最为重要。 “这意味着我们现在有了设计改性药物的信息，这些药物将更有效地运作。”

第二项研究与弗林德斯大学合作，使用 X 射线晶体学首次证明了一种潜在的新药，rivoglitazone 与 PPARγ受体结合。rivoglitazone 完全激活 PPARγ，但比其他具有类似作用效果的药物副作用更少。

来自弗林德斯大学医学院（现在的 La Trobe 大学）的 Rajapaksha 博士说：“显示这种化合物如何与其靶标相互作用是迈向能够设计具有更高效率和更少副作用的新疗法的关键一步。 “结构性信息的缺乏妨碍了确定所涉及的精确机制。”

六、总局关于药物临床试验数据自查核查注册申请情况的公告（2017年第59号）2017-04-11　米内网

国家食品药品监督管理总局决定对新收到44个已完成临床试验申报生产或进口的药品注册申请（见附件）进行临床试验数据核查。现将有关事宜公告如下：

一、在国家食品药品监督管理总局组织核查前，药品注册申请人自查发现药物临床试验数据存在真实性问题的，应主动撤回注册申请，国家食品药品监督管理总局公布其名单，不追究其责任。

二、国家食品药品监督管理总局食品药品审核查验中心将在其网站公示现场核查计划，并告知药品注册申请人及其所在地省级食品药品监管部门，公示10个工作日后该中心将通知现场核查日期，不再接受药品注册申请人的撤回申请。

三、国家食品药品监督管理总局将对药物临床试验数据现场核查中发现数据造假的申请人、药物临床试验责任人和管理人、合同研究组织责任人从重处理，并追究未能有效履职的食品药品监管部门核查人员的责任。

特此公告。

附件：44个药物临床试验数据自查核查注册申请清单

食品药品监管总局

2017年5月16日

2017年第59号公告附件.xlsx

七、政策门槛有望再放宽！医药电商的未来好着呢

2017-05-17 米内网

踏入 2017 年，随着新医改政策的不断推进，行业进入了政策落地的时刻，两票制、营改增、一致性评价等政策暴风骤雨式的推进，让行业面临新的机遇与挑战。

在医药电商领域，第三方平台售药行为的停止与证照审批的取消，及近日网传国家卫生计生委办公厅印发的《关于征求互联网诊疗管理办法（试行）（征求意见稿）和关于推进互联网医疗服务发展的意见》，更是改变了行业的格局与准入门槛，如何摸准医药电商领域的政策脉搏，找准行业风口，时刻触动着行业从业者的神经。

《新环境、新策略、新机遇》医药电商主题论坛

为满足行业发展需求，《21 世纪药店》于 5 月 16 日上海药交会开幕首日，举办主题为《新环境、新策略、新机遇》的医药电商主题论坛，通过详细论述行业环境、企业策略、未来机遇这三大层面，剖析医药电商行业未来的发展趋势。本次会议为参会嘉宾呈现了哪些精彩观点？未来医药电商行业趋势又将有哪些变化？

政策门槛再次降低？

作为南方医药经济研究所的下属媒体，《21 世纪药店》长期关注、研究药品零售行业不同渠道发展。在 2016 年，为了满足医药电商从业者的政策了解需求，发挥第三方行业平台应有的作用，《21 世纪药店》更是组建中国医药电商专委会，通过组织行业会议，发布行业监测报告，分享行业先进经营模式，以促进医药电商的长远发展。

《21 世纪药店》总经理邵旭东

《21 世纪药店》总经理邵旭东就在本次主题论坛上致辞表示：“经过去年一年的努力，电商专委会的工作受到行业的广泛好评，为了继续推动医药电商的发展，我们在 2017 年将继续举办各种类型的电商活动，为行业提供多元化的服务。”

与 2016 年的电商系列论坛一样，本次论坛的开篇环节，无疑是行业人士最为关注的医药电商监测报告。据报告嘉宾，南方医药经济研究所网监中心高级研究员张涛表示，在 2017 年

第一季度，虽然在国务院的要求下，大多数地区的医药电商证照审批工作已经暂停，等待新政策的出台，然而由于目前仍没有具体指导文件，因此部分地区依然对申请企业批发证照，促使医药电商数量的持续上涨。

“仅仅在 2017 年第一季度，就新增《互联网药品交易服务资格证》54 个，拥有电商运营资质的企业总数达到 921 家，再创行业新高。”张涛说。

据张涛推测，随着电商资质审批的放开及管理办法的改变，行业门槛将进一步降低。据介绍，在 4 月 5 日，相关部门拟定了《网络药品经营监督管理办法》的初稿，提到未来医药电商的 ABC 证不仅取消审批，甚至可能连备案都不用，只需要向地方局告知登记即可。同时，单体药店亦有可能开放网售资格。“虽然这只是初稿，但医药电商政策门槛不断降低的信号已经十分明显，未来将有越来越多企业进入这一领域进行多种创新。”张涛如此判断。

前景无限，从构建服务闭环开始

随着近年以康爱多、七乐康、健客、1 药网等为首的品牌医药电商的快速崛起，不少业内人士开始抛出医药电商已经进入下半场的概念。面对接下来新一轮医药电商的大爆发，行业先机真的已经被完全抢占了吗？

平安好医生副总裁汪坤

对于这个问题，平安好医生副总裁汪坤给出了否定的答案。他以小红书、网易考拉这两家电商企业的崛起为案例，表明即便在以 BAT 为首的电商巨头已经涉足的情况下，创新型企业依

然有突围空间，更遑论市场成熟程度远远比不上大零售渠道的医药电商领域了。“尤其是在各品牌企业市场占有率并不高的环境下，医药电商离下半场依然有一段时间距离。”汪坤如此说道。

百度外卖医疗业务部总经理邵清

在市场未来空间依然可期的情况下，企业又应如何抓住发展机会？行业资深专家，百度外卖医疗业务部总经理邵清就在论坛上通过发表《从医到药，医药电商的医药结合之路》主题报告，全面分析了医药电商的前世今生与未来趋势。在邵清看来，在互联网技术不断创新的环境下，医药产业互联网化将持续深入，行业运营模式亦将不断创新，医药电商企业要找准风口，必须实现全产业链布局。“因为，未来 30 年将是产业互联网化的天下。”邵清如此说道。

广东健客医药有限公司 CEO 谢方敏

除了预测行业趋势外，本次论坛还特邀广东健客医药有限公司 CEO 谢方敏，通过讲解健客近年来慢病管理的尝试，为行业提供最接地气的医药电商经营模式。据谢方敏介绍，对于慢病管理，医药电商拥有天然的品类优势、平台属性优势、数据优势与综合服务优势，能有效提升患者用药依从性，实现定时的服药提醒、专业的用药指导及深入健康管理，建立完善的慢病管理体系。

“当然，慢病服务仅是医药电商进行模式创新，实现专业服务的其中一个领域，正如邵清所言，未来医药电商要成功突围，就必须建立自身的服务闭环，健客在此方面也有自身的思考，我们的定位是成为最值得信赖的智慧健康服务平台，因此我们的战略布局将囊括医药、医疗等多个领域，打造以医药电商作为切入点，发展移动医疗等多健康服务，通过整合多方资源，实现智慧健康的产业闭环。”谢方敏最后总结道。

互联网医疗将何去何从

除分享主题报告外，本次论坛针对近日行业热议的《关于征求互联网诊疗管理办法（试行）（征求意见稿）和关于推进互联网医疗服务发展的意见》，亦邀请上下游企业代表进行现场互动讨论，他们对于《意见》的发布，分别进行了不同的解读。

（从左到右：谢方敏、汪坤、司杰、张锦桦、杨伟雄）

广东健客医药有限公司 CEO 谢方敏：

《意见》的出台将有效推动医药电商与互联网医疗产业的融合与升级。毕竟在此之前，业内一直缺乏明确的指导文件，《意见》的出台意味着互联网医疗终于获得应有的合法性地位，

这一方面将使各地区的监管部门有了互联网医疗监管的执行方向，促使更多互联网医疗落地，另一方面亦为从业者注入了信心，为医药电商与互联网医疗的结合奠定了基础。

平安好医生副总裁汪坤：

《意见》所提及的管理方式固然提高了行业运营成本与准入门槛，但亦带来了正面作用，其将有效排除了浑水摸鱼者的进入，确保了行业的有序发展，使真正想在互联网医疗发展的同仁更能静下心来研究可行的互联网医疗落地模式。

百度外卖医药健康业务部总经理邵清　：

《意见》的公布虽然并不代表最终监管法案，但已经释放出明确的信号，未来要经营互联网医院，企业必须有自身的实体医院，其实这也可以理解，毕竟医药电商的经营前提也是连锁药店，其发布存在合理性。

西安利君制药有限公司常务副总经理司杰：

互联网医疗必须与线下实体医疗机构结合的概念，体现了政策对人基本权利的尊重，毕竟医疗是一件严肃的工作，面对面诊疗依然是让患者最为放心的做法。但无可否认的是，在技术的不断革新下，互联网医疗仍然是行业发展的趋势，《意见》的出台虽然在短时间内建立了行业壁垒，减低了“野蛮人”的进入速度，但行业人士要真正立于不败之地，仍需不断探索互联网技术的应用，实行自我革命，才能赢得长远的市场空间。

万年青控销事业部总经理杨伟雄：

互联网技术的应用，解决的是行业长期存在的信息不对称问题，无论在医药还是医疗领域，其未来都大有可为，尤其是互联网医疗的出现，在政策鼓励医生多点执业的背景下，更是加快了医生社会化的进度，倒逼传统医疗体制改革，对行业及患者都有极大的意义。

浙江英特药业有限责任公司副总经理、浙江英特药业有限责任公司电子商务分公司总经理张锦桦：

《意见》的出台有利有弊，互联网医疗的合法性虽然得到确认，但在条条框框的约束下，如医生需要在互联网医院定点执业并获得原医院领导同意、互联网医院无法实现首诊等，都限制了互联网医疗的发展机会。行业要真正实现大爆发，需坚持与监管部门沟通，让互联网医

疗的执业医生能够摆脱原有的体制真正获得监管部门的独立认可，以有别于线下医院的模式出现，才能真正倒逼医疗体制改革。

八、三生制药获得礼来胰岛素产品优泌林在中国大陆的分销和推广权利

2017-05-17　美通社

16 日，三生制药宣布，已经与礼来中国达成战略合作协议，自 2017 年 7 月 1 日起，获得礼来旗下的胰岛素产品优泌林®在中国独家经销和推广的权利。

根据协议，三生制药将利用其全国销售网络，并基于现有代谢产品事业部的基础上，组建更具有广泛覆盖规模的糖尿病产品(包含优泌林®)市场和推广团队。礼来制药负责按照其全球质量标准进行生产及供应优泌林®。目前，双方正在密切合作以确保实现平稳过渡。

优泌林®诞生于 1982 年，是世界上第一个人工基因合成的人胰岛素产品，也是全球首个应用重组 DNA 技术创造的人用医疗产品。自 1997 年在中国上市以来，已累计服务数千万中国糖尿病患者。

三生制药董事局主席兼首席执行官娄竞博士对此次合作的前景十分看好，他表示：“礼来是全球领先的制药企业，与礼来就优泌林®这一经典人胰岛素产品的推广和分销权达成的战略合作，将进一步丰富三生现有的产品线，惠及更多的中国患者。三生制药作为中国生物制药行业的先锋，在研发、生产及营销生物技术产品方面拥有丰富的经验和成熟的网络。三生制药将扩大代谢产品专属市场和营销团队，利用原有经验更加迅速地服务中国更广阔基层市场，让更多患者有机会接触到优质产品。与礼来的这次战略合作，代表三生制药全面进军糖尿病治疗领域，让一家本土化的优秀企业为中国的糖尿病患者带来更多福祉，实现社会、患者等多方共赢。”

礼来中国总裁贺安德(Andrew Hodge)先生表示：“与三生制药达成的协议是礼来与中国本土领先制药企业的又一成功的合作案例。一方面通过合作伙伴遍布全国的销售网络，优泌林®能够惠及更广泛的中国糖尿病患者;同时，此次合作也使礼来能够集中优势资源专注于优泌乐®系列胰岛素类似物产品和即将上市的糖尿病领域的其它新药。为更好地服务糖尿病

患者，礼来还将继续加大在华投资力度。例如，礼来斥资 30 亿元人民币对苏州胰岛素生产基地的升级工作即将完成。”

糖尿病流行对人类健康和社会经济产生重大影响，尤其是在发展中国家。糖尿病的并发症可导致心血管病变、肾功能衰竭、失明、截肢等。世界卫生组织于 2017 年 4 月 6 日首次发布《全球糖尿病报告》，显示全球糖尿病成年人患者近 40 年内增加了 3 倍，其中多数生活在发展中国家。据统计，中国 2 型糖尿病发病率在过去数十年中呈“爆炸式”增长，1980 年只有不到5%的中国男性患有糖尿病，而目前这一比例超过了10%(2015年病患人数高达1.1亿人)，中国现已成为全球糖尿病患者第一大国。

三生制药成立于 1993 年，是以科技创新为长期目标的中国生物技术领军企业。多年来，凭借其丰富的学术推广经验和优秀的管理团队，所涉足的产品领域，都无可争议的成为市场绝对领导者。三生有能力将礼来这一经典品牌迅速地覆盖中国广阔市场。此次与礼来中国的战略合作，使广大患者获得了在糖尿病治疗领域的更多优选方案，实现了该产品与三生制药现有代谢团队的协同效应。三生制药将持续致力于实现“以高品质的药品提高患者生存质量，为人类健康造福”的崇高使命，为中国糖尿病患者及该治疗领域的学术进步与发展做出重要贡献。

九、市场放开 乐普医疗等企业大举买中小医院！

2017-05-17 北京商报

在医疗资源下沉、医疗市场大放开的背景下，医药企业开始收购中小医院，为调整业务、完善产业链而布局。

记者统计发现，朗玛信息、乐普医疗、恒康医疗、济民制药等多家上市公司均大举并购三级以下医院。分级诊疗、社会办医等利好政策频出，吸引国内制药大佬频频涉足包括医院在内的健康服务业终端。目前，成功案例却少之又少，企业收购医院后面临品牌建设、内部经营管理、医生资源配置等问题。

基层医院受热捧

2017 年是医改大年，分级诊疗、一致性评价、两票制等政策持续推进，倒逼医药行业重构。

今年《政府工作报告》中明确提出启动多种形式医疗联合体建设试点，三级公立医院要全部参与并发挥引领作用，建立促进优质医疗资源上下贯通的考核及激励机制，增强基层服务能力。利好政策吸引下，众多医药企业收购终端基层医院完善产业链。

三级医院资源不足、大部分基层医院资源闲置的现状下，县一级医院被认为有较大发展机遇。

卫计委相关统计数据显示，2015 年三级医院“诊疗人次/机构”增速首次为负;2015 年县级(含县级市)医院的“诊疗人次/机构”仍然保持增速为正，县级医院在分级诊疗中的作用开始显现。

记者注意到，从 2015 年下半年开始，朗玛信息、恒康医疗等多家上市公司开始并购三级以下医院。

2015 年 11 月，贵阳市政府批复同意贵阳六院改制为营利性医疗机构。根据方案，贵阳六院所有资产、业务和人员，由贵阳市医院管理(集团)有限公司下设六医公司承接，六医公

司引入社会资本，朗玛信息以现金方式增资控股，持有 66%股权;2015 年 11 月，恒康医疗宣布收购盱眙恒山中医院全部股权;2016 年 3 月，乐普医疗全资收购洛阳市第六人民医院;2016 年 12 月，济民制药以 3.44 亿元的总价收购并增资鄂州二医院获得 80%的股权。

完善产业链

布局终端基层医院是企业布局大健康产业链不可或缺的环节。

乐普医疗收购洛阳六院时表示，洛阳六院将作为该公司未来三级心血管网络医院服务体系中区域性心血管网络医院的载体之一，有助于促进该公司“建设二三线城市心血管专科医院群”目标实现，进一步完善乐普医疗全产业链大健康生态圈建设。

一位不愿具名的分析人士表示，做支架等传统医疗器械起家的乐普医疗一直想打造心血管产业链平台。当前，支架价格处于下滑状态，利润率越来越薄致使乐普医疗寻找新的利润增长点。

济民制药是一家大输液公司，受国家医保控费、限制抗生素使用以及限制或取消门诊输液政策影响，公司业绩持续下滑。目前，国内输液产品 60%-70%仍主要集中在低端普通输液领域，优化产品结构成为济民制药当务之急。

恒康医疗副总裁、医院管理中心总经理王伟此前在接受媒体采访时表示，通过收购区域医疗中心实现医疗服务为核心的大健康战略，并增强综合优势。2016 年上半年业绩上升主要原因包括瓦房店三医院、盱眙县中医院和福源医院业绩合并到报表范围。

收购面临挑战

收购基层医院对企业布局区域医疗服务有重要意义。分析认为，收购公立医院具有承接优良医疗服务项目、医疗管理体系、医疗客户资源以及培养医疗服务人才等优势。不过，目前政策对于公立基层医院改革较为模糊，公立医院收购过程缓慢，历时较长，收购失败的可能性较大。

罗兰贝格咨询合伙人金毅曾表示，一系列医改政策推动，多元医院产权结构正在建立过程中，社会资本也在积极布局，“体制内非核心的公立医院”、“公立医院集团通过并购做实”等或将成为下一步并购方向。

上述不愿具名分析人士表示，虽然国内很多企业都在收购医院，但是大部分企业面临着收购后如何进行管理的问题。

“收购后的医院品牌建设、医院内部经营管理以及医院医生资源配置等都成为摆在收购企业面前的难题。目前，国家关于公立医院改革并没有具体的措施出来，企业不要茫然跟风收购民营或效果不太理想的公立医院。“国家一旦出了具体政策措施，会试点推进，到时候会拿出经营效果较好甚至是三甲医院来让社会资本进入。”

十、O2O 进入退烧期，这家药企为何还要“勇往直前”

2017-05-19　米内网

最近，山东步长制药股份有限公司对外发布公告，拟以 6000 万元的价格认购北京快方科技有限公司(以下简称“快方送药”)276,666.67 元的新增注册资本，本次增资完成后，山东步长将持有快方送药 11.538%的股权。

对于此次投资，山东步长表示是为了实施公司在终端市场延伸布局的战略。据了解，快方送药在 2016 年转型，其运作模式从原来“一小时送药上门服务”的第三方配送服务商转为全自营药店，目前在北京已拥有 18 家门店。

受相关政策因素影响，越来越多的药企布局 OTC 市场。山东步长相中转向自开药店的快方送药，这种方式能否成为药企切入药品零售终端的主流?

为什么相中快方送药?

O2O 送药模式曾受到许多电商和连锁药店的热捧，但由于配送成本问题，“最后一公里”让不少试水者纷纷折戟。

从 2014 年 9 月快方送药 APP 上线至今，共获得四轮融资：2014 年 9 月，获九合创投数百万元天使投资;2015 年 7 月，完成 5000 万元 A 轮融资;同年 10 月，获天图资本 2 亿元 B 轮融资;2017 年 4 月，获山东步长 6000 万元 B+轮融资。

耐人寻味的是，据山东步长的公告显示，截至 2016 年 12 月 31 日，2016 年度快方送药的营业收入为 4281.66 万元，净利润为-4706.64 万元。那么，在医药 O2O 进入退烧期的背景下，吸引山东步长的是什么?

经过两年多的努力，快方送药已形成较成熟的一键购药、快速拣药、智能订单分配和 Lbs 实时定位四大系统，并且研发出无线温湿度配送箱。有报道称，快方送药的准时送达率 95%以上，库存准确率达 90%以上，服务好评率为 99%以上。不过，只要有钱可烧，估计这些系统其他电商也可以建立，因此对于山东步长而言，这应该不是“动心”的关键理由。

快方送药在 2016 年转型为全自营药店模式，即开设实体药店自营。以北京为例，18 家门店以 50 公里为半径，覆盖了北京五环以内的区域，能在一小时内送达区域内的任何一个角落。快方送药的服务范围除了北京，还涵盖上海、杭州、广州等多个城市。截至 2016 年 6 月，快方送药的自营药店全部升级改造完成，日配送能力超过 5 万单。

如果不断有资本加入，快方送药这种综合了电商和实体店功能的门店数量可以继续增加，扩大配送范围。显然，这与山东步长“在终端市场延伸布局”的战略是相符的。

为什么选择这种路径?

此次并不是山东步长首次投资医药电商企业，其子公司山东丹红制药曾投资七乐康。有消息称，七乐康计划成立银川七乐康药业连锁公司，从而打通线上线下的医疗、药品服务。

近几年来，有志进军药品零售终端的大型药企不少，代表企业有天士力、仁和药业、白云山等。2015 年 11 月，天士力与泰康人寿、中原银行共同出资成立“大健康产业基金”，分别于 2016 年 9 月和 11 月投资甘肃的众友健康和山东的立健;2016 年 10 月，仁和药业拟采用定增方式募资 12 亿元，用于收购 2000 家门店;2017 年 1 月，广州白云山发布公告称，拟出资 8 亿元获得一心堂的 6.92%的股权，成为第三大股东。

药企自建药品零售终端之举，普遍不被零售行业看好。天士力虽然旗下有连锁药店，但多年来主要在东北发展，而选择区域连锁的佼佼者如众友健康和立健，则能补上覆盖区域少的短板。经过战略调整的一心堂又将启动快速扩张的战车，业内人士推测，白云山与一心堂“联姻”，有利于促进其产品在西南市场的销售。

当然，山东步长也可效仿天士力，选择投资一些有发展潜力的区域连锁，不过不利因素有三，一是较有实力和潜力的区域连锁如新兴、一树等皆名花有主;二是所需的投入较大，动辄上亿元;三是竞争对手已抢占先手，后来者难以居上。

“弯道超车”的最佳时机是模式换代，在医药新零售时代，这样的机会已经到来，无论是生产企业还是药店，其模式都需要转型升级，从未来的发展来看，拥抱互联网的药店将会拥有更多的机遇。

据此推测，山东步长看好的是快方送药的未来。

会不会有更多的追随者?

近几年，一些在新三板上市、规模在几千万元到两亿元之间的区域连锁，纷纷调整战略中心，转向做电商。之所以做出这样的选择，是因为即使是在新三板上市，融到的资金也有限，无法在门店规模上迅速做大，成为区域的主导者。但转向电商则不然，起码在这条新的“跑道”上有“弯道超车”的可能。

从药企的角度来看，道理亦然。收购药店或投资大型连锁药店花费不菲，而且上市连锁和一些区域连锁的并购已进行了几年，有价值、合适的合作对象数量大幅度减少。不过，投资拥抱互联网的新型连锁药店则不然，假以时日，现在的年轻群体必会成为网上购药的主力军，届时新型的连锁药店的前途不可限量。

倘若就此断定今后会有更多的药企效仿山东步长，为时过早。“电商”是“烧钱”的行业，可不等于永远“烧”下去，在前期“烧钱”阶段占领市场份额的任务完成后，终究要想方设法实现盈利，否则将遭到投资者舍弃。转型后的快方送药能不能扭亏为盈，需要市场和时间来验证。

事实证明，在互联网时代，实体店的作用不但没有减弱，反而更加突出。不同的是，与传统的药店相比，互联网时代药店的功能发生了变化，药企和药店的合作与营销模式也需相应改变。

十一、Amgen Submits Biologics License Application To The FDA For Erenumab

（安进向美国 FDA 提交潜在重磅偏头痛药物 erenumab 上市申请）

2017-05-18 Issued by PRNewswire

Erenumab is an Investigative Treatment Specifically Designed to Prevent Migraine
Only Molecule in Late-Stage Development to Directly Target the Calcitonin Gene-Related Peptide Receptor
Amgen and Novartis to Co-Commercialize Erenumab in the U.S.

THOUSAND OAKS, Calif., May 18, 2017 /PRNewswire/ -- Amgen (NASDAQ:AMGN) today announced the submission of a Biologics License Application (BLA) to the U.S. Food and Drug Administration (FDA) for erenumab to prevent migraine. Erenumab is specifically designed to prevent migraine by blocking the Calcitonin Gene-Related Peptide (CGRP) receptor. This BLA includes data from pivotal studies in patients with episodic and chronic migraine.

"People with migraine lose a substantial part of their lives enduring or managing the disease, which takes time away from their loved ones, social activities and workplace responsibilities. Approximately 3.5 million Americans currently take a preventive treatment to reduce their number of migraine days, yet 80 percent of those discontinue these treatments within one year," said Sean E. Harper, M.D., executive vice president of Research and Development at Amgen. "This application represents a long-awaited step towards addressing the unmet need faced by the migraine community through the potential delivery of erenumab, which has demonstrated strong efficacy, safety and tolerability in patients with episodic and chronic migraine."

The erenumab global clinical trial program has enrolled more than 2,600 patients experiencing four or more migraine days per month, with some patients receiving erenumab for up to three years. Results from the chronic migraine study were published in *The Lancet Neurology* in April 2017 and detailed results from Phase 3 in episodic migraine have been submitted for publication.

Results from the pivotal studies, investigating the efficacy of erenumab versus placebo in reducing the number of migraine days for patients with episodic and chronic migraine, will be presented during the 59th Annual Scientific Meeting of the American Headache Society in Boston this June.

十二、GSK announces NEJM publication of positive phase III study investigating mepolizumab in patients with Eosinophilic Granulomatosis with Polyangiitis (EGPA) (GSK 宣布抗 IL-5 单抗类抗炎药 Nucala（mepolizumab，美泊利单抗）治疗复发性和难治性嗜酸性肉芽肿血管炎（EGPA）的一项关键性 III 期临床研究的积极数据已在线发表于医学顶级期刊《新英格兰医学杂志》（NEJM）。)

2017-05-17 Issued by GSK

GSK today announced publication in the *New England Journal of Medicine*, of a randomised, double-blind, placebo controlled study investigating the efficacy and safety of mepolizumab, an IL-5 antagonist, vs placebo as an add-on therapy in patients with relapsing and/or refractory Eosinophilic Granulomatosis with Polyangiitis (EGPA). EGPA is a rare disease characterised by widespread inflammation in the walls of small blood vessels (vasculitis) which may affect multiple organ systems and be associated with fatigue, fever and weight loss.

The study was a collaboration between GSK and the National Institute of Allergy and Infectious Diseases (NIAID), part of the U.S. National Institutes of Health (NIH). Headline data from the study were previously announced in November 2016.

Efficacy results

In the 52-week pivotal phase III study, mepolizumab treatment demonstrated efficacy based on both co-primary efficacy endpoints and all secondary endpoints. Treatment with mepolizumab was in addition to standard of care (glucocorticoids with or without immunosuppressants).

Patients treated with mepolizumab had a significantly greater accrued time in remission (defined as a prednisolone/prednisone dose of ≤4mg/day and a Birmingham Vascular Activity Score = 0) over the 52-week treatment period compared to placebo, with 28% of patients on mepolizumab achieving remission for at least 24 weeks versus 3% on placebo ($p<0.001$). In addition, a higher proportion of patients in the mepolizumab group were in remission at both Weeks 36 and 48 compared to the placebo group (32% versus 3%; $p<0.001$).

More patients treated with mepolizumab achieved remission within the first 24 weeks of the study and remained in remission until Week 52 compared to those receiving placebo (19% versus 1%; $p=0.007$). Over 52 weeks, time to first relapse was significantly longer for patients on mepolizumab ($p<0.001$); time to first major relapse was also longer for patients treated with mepolizumab compared to placebo ($p=0.042$). Patients treated with mepolizumab were also able to achieve significantly lower average doses of prednisolone/prednisone during Weeks 48 to 52 compared to placebo, with 44% able to taper their dose to ≤4mg/day versus 7% on placebo ($p<0.001$).

A total of 47% of mepolizumab-treated patients versus 81% of placebo-treated patients did not achieve protocol-defined remission. Approximately half the participants in the mepolizumab

group had a relapse, nonetheless a 50% lower rate of relapse with mepolizumab treatment was seen than was observed with placebo in this trial (1.14 per year for the mepolizumab group versus 2.27 per year for the placebo group), which highlights the high morbidity and challenge of managing patients with this progressive disease.

Safety results

There was no difference between the two treatment groups in the proportion of patients experiencing on-treatment adverse events (97% versus 94%) and overall the adverse event profile for mepolizumab was similar to that seen in previous studies with no new safety signals observed. Fewer patients in the mepolizumab group reported serious adverse events versus those in the placebo group (18% versus 26%), with the most frequently reported being asthma worsening/exacerbation (3% versus 6%). Systemic reactions were infrequent and reported with higher incidence in the mepolizumab group compared to placebo. One death was reported in a patient receiving mepolizumab which was not considered by the investigator to be related to study treatment.

Dr. Michael E Wechsler, Professor of Medicine at National Jewish Health in Denver, Colorado, US, Principal Investigator and lead author of the study said: "EGPA patients suffer from recurrent relapses that place these patients at greater risk of permanent tissue and organ damage. There are currently no therapies specifically approved for EGPA. While systemic corticosteroids form the cornerstone of EGPA treatment, they can be associated with significant side effects. In this study, mepolizumab met several important clinical goals in the treatment of EGPA: it increased accrued time in remission, reduced frequency of relapse and exacerbation, and enabled patients to reduce corticosteroid dose. These data confirm the potential of mepolizumab as a future treatment option for patients with this rare disease."

Steve Yancey, Vice-President and Medicines Development Lead for mepolizumab, GSK, commented: "Uncontrolled eosinophilic inflammation leads to an unpredictable condition for patients who often fear the relapses that are a common feature of the disease. Our goal is to provide patients and healthcare professionals with a new treatment option to help control this disease. The success of this study is a testament to the hard work of the GSK team, the NIAID, and the investigators, and we thank all the patients whose participation enabled the study to proceed."

Results of this study in patients with relapsing and refractory EGPA will support GSK's plans to submit regulatory applications, expected later in 2017.

Mepolizumab is not approved for use anywhere in the world for EGPA.

Results of the study are available at:
http://www.nejm.org/doi/full/10.1056/NEJMoa1702079?query=featured_home

In addition, full results of the study are being presented during the American Thoracic Society meeting:

- 15-11.15am 21st May - *NEJM-JAMA session*: *Discussion on the Edge: Reports of Recently Published Pulmonary Research*

- 15-11.15am 22nd May - *Oral presentation*: *Mepolizumab for the Treatment of Patients with Eosinophilic Granulomatosis with Polyangiitis: A Phase III Randomized, Placebo-Controlled Trial.*

About the study

The pivotal phase III study was a randomised, double-blind placebo controlled study designed to investigate the efficacy and safety of mepolizumab 300mg (administered subcutaneously every 4 weeks) compared to placebo, in patients already receiving standard of care, over a 52-week study treatment period. The study was conducted in 31 academic centres and hospitals across nine countries and enrolled 136 patients (mepolizumab n = 68; placebo = 68) with relapsing or refractory EGPA receiving standard of care therapy (i.e. treatment for more than four weeks on stable dose prednisolone/prednisone ≥7.5mg - ≤50mg day) with or without immunosuppressive therapy. The study population was representative of patients with EGPA treated with glucocorticoids with or without additional immunosuppressant therapy.

Study endpoints

The co-primary endpoints were the total accrued weeks of remission over the full trial period, and the proportion of patients in remission at both Weeks 36 and 48.Remission was defined by a Birmingham Vasculitis Activity Score (BVAS), a scoring system to assess disease activity, of zero, and a prednisolone/prednisone dose ≤4mg/day.

The study included six secondary endpoints investigating relapse, remission and corticosteroid use, all considered clinically relevant for patients with EGPA.

About EGPA (previously known as Churg-Strauss Syndrome)

Eosinophilic granulomatosis with polyangiitis (EGPA) is a rare disease that causes inflammation of the small blood vessels (or vasculitis). The global incidence is generally reported to be in the range 1–4 per million, with an estimated prevalence of approximately 14–45 per million. The mean age of diagnosis is 48 years and the disease can be life-threatening for some patients.

In EGPA, patients usually develop asthma initially, before the vasculitis extends to inflammation of the small blood vessels that supply tissues in the lungs, sinuses, skin, nerves and other organs. EGPA can result in damage to almost every organ in the body and the symptoms common to most include extreme fatigue, weight loss, muscle and joint pain, sinonasal symptoms and breathlessness, all of which affect patients' ability to carry out everyday activities without difficulty.

The current approach to disease management is primarily based on reduction of active inflammation and suppression of the immune response through the use of corticosteroid therapy and concomitant immunosuppressive therapy (e.g., methotrexate, azathioprine, mycophenolate

mofetil) and/or cytotoxic agents (e.g., cyclophosphamide). Although the use of these treatments can be effective for establishing remission, patients remain vulnerable to either the complications of the long-term use of these therapies, or to the risk of relapse, particularly if the dose of corticosteroid is reduced.

About mepolizumab

Mepolizumab is a humanised IgG monoclonal antibody specific for interleukin 5 (IL-5). IL-5 is a cytokine which regulates the growth, activation and survival of eosinophils (white blood cells) and provides an essential signal for the movement of eosinophils from the bone marrow into the lung and other organs. Mepolizumab binds to human IL-5, stopping it from binding to its receptor on the surface of eosinophils. Inhibiting IL-5 binding in this manner reduces blood, tissue and sputum eosinophil levels, which in turn reduces eosinophil-mediated inflammation.

Mepolizumab is not approved anywhere in the world for EGPA. Mepolizumab is approved for use in the E.U., under the brand name Nucala, for use as an add-on treatment for severe refractory eosinophilic asthma in adult patients. Nucala is approved for use in the U.S. as an add-on maintenance treatment of patients with severe asthma aged 12 years and older, and with an eosinophilic phenotype. It is not approved for the treatment of other eosinophilic conditions or relief of acute bronchospasm or status asthmaticus.

Nucala has also been approved in Canada, Australia, Japan, Switzerland, Chile, South Korea and Taiwan.Trade marks are owned by or licensed to the GSK group of companies.

Important Safety Information for Nucala in Severe Eosinophilic Asthma

The following information is based on the US Prescribing Information for Nucala. Please consult the full Prescribing Information for all the labelled safety information for Nucala.

CONTRAINDICATIONS

Nucala should not be administered to patients with a history of hypersensitivity to mepolizumab or excipients in the formulation.

WARNINGS AND PRECAUTIONS

Hypersensitivity Reactions

Hypersensitivity reactions (e.g. anaphylaxis, angioedema, bronchospasm, hypotension, urticaria, rash) have occurred following administration of Nucala. These reactions generally occur within hours of administration but in some instances can have a delayed onset (i.e. days). In the event of a hypersensitivity reaction, Nucala should be discontinued.

Acute Asthma Symptoms or Deteriorating Disease

Nucala should not be used to treat acute asthma symptoms, acute exacerbations, or acute bronchospasm.

Opportunistic Infections: Herpes Zoster

In controlled clinical trials, 2 serious adverse reactions of herpes zoster occurred in subjects treated with Nucala compared to none in placebo. Consider varicella vaccination if medically appropriate prior to starting therapy with Nucala.

Reduction of Corticosteroid Dosage

Do not discontinue systemic or inhaled corticosteroids (ICS) abruptly upon initiation of therapy with Nucala. Decreases in corticosteroid doses, if appropriate, should be gradual and under the direct supervision of a physician. Reduction in corticosteroid dose may be associated with systemic withdrawal symptoms and/or unmask conditions previously suppressed by systemic corticosteroid therapy.

Parasitic (Helminth) Infection

It is unknown if Nucala will influence a patient's response against parasites. Treat patients with pre-existing helminth infections before initiating therapy with Nucala. If patients become infected while receiving treatment with Nucala and do not respond to anti-helminth treatment, discontinue treatment with Nucala until infection resolves.

ADVERSE REACTIONS

The most common adverse reactions (≥3% and more common than placebo) reported in the first 24 weeks of two clinical trials with Nucala (and placebo) were: headache, 19% (18%); injection site reaction, 8% (3%); back pain, 5% (4%); fatigue, 5% (4%); influenza, 3% (2%); urinary tract infection 3% (2%); abdominal pain upper, 3% (2%); pruritus, 3% (2%); eczema, 3% (<1%); and muscle spasm, 3% (<1%).

Systemic Reactions, including Hypersensitivity Reactions: In 3 clinical trials, 3% of subjects who received Nucala experienced systemic (allergic and nonallergic) reactions compared to 5% in the placebo group. Systemic allergic/hypersensitivity reactions were reported by 1% of subjects who received Nucala compared to 2% of subjects in the placebo group. Manifestations included rash, pruritus, headache, and myalgia. Systemic nonallergic reactions were reported by 2% of subjects who received Nucala and 3% of subjects in the placebo group. Manifestations included rash, flushing, and myalgia. A majority of the systemic reactions were experienced on the day of dosing. Reports of anaphylaxis have been received postmarketing.

Injection site reactions (e.g. pain, erythema, swelling, itching, burning sensation) occurred at a rate of 8% in subjects treated with Nucala compared with 3% in subjects treated with placebo.

USE IN SPECIFIC POPULATIONS

The data on pregnancy exposures from the clinical trials are insufficient to inform on drug-associated risk. Monoclonal antibodies, such as mepolizumab, are progressively transported across the placenta in a linear fashion as pregnancy progresses; therefore, potential effects on a foetus are likely to be greater during the second and third trimesters of pregnancy.

GSK – one of the world's leading research-based pharmaceutical and healthcare companies – is committed to improving the quality of human life by enabling people to do more, feel better and live longer. For further information please visit www.gsk.com/about-us/.

Cautionary statement regarding forward-looking statements

GSK cautions investors that any forward-looking statements or projections made by GSK, including those made in this announcement, are subject to risks and uncertainties that may cause actual results to differ materially from those projected. Such factors include, but are not limited to, those described under Item 3.D 'Principal risks and uncertainties' in the company's Annual Report on Form 20-F for 2016。

十三、Brodalumab receives positive CHMP opinion for the treatment of adult patients with moderate-to-severe plaque psoriasis (Brodalumab 获欧盟 CHMP 支持批准，治疗斑块型银屑病)

2017-05-19 Issued by AstraZeneca

AstraZeneca today announced that its partner LEO Pharma has received a positive opinion from the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) recommending the approval of brodalumab for the treatment of adult patients with moderate-to-severe plaque psoriasis who are candidates for systemic therapy.

Brodalumab is the first and only fully human monoclonal antibody that selectively targets the IL-17 receptor. By binding to the receptor, brodalumab effectively blocks the biological activity of several pro-inflammatory IL-17 cytokines, which are important in psoriasis, a chronic, debilitating skin disease that causes red patches of skin covered with silvery scales.

In July 2016, AstraZeneca announced an agreement granting LEO Pharma, a specialist in dermatology, exclusive rights to develop and commercialise brodalumab in Europe. Today's announcement follows the approval of brodalumab by the US Food and Drug Administration (FDA) (brand name *Siliq*) in February 2017 and the approval by the Japanese Pharmaceuticals and Medical Devices Agency, in 2016.

The CHMP's positive opinion on brodalumab will now be reviewed by the European Commission, which has the authority to approve medicines for the European Union (EU). The final decision is applicable to all EU and European Economic Area countries (Iceland, Liechtenstein and Norway).

十四、Novartis receives positive CHMP opinion for first-line use of Zykadia® in ALK-positive advanced non-small cell lung cancer (NSCLC)（诺华抗癌药 Zykadia 进入 ALK 阳性肺癌一线治疗）

2017-05-19 Issued by Novartis

- Phase III trial, first-line treatment with Zykadia resulted in improved progression-free survival (PFS) over SOC chemotherapy with maintenance, including in patients with brain metastases[1]
- Zykadia is currently approved in the European Union (EU) for the treatment of adult patients with ALK-positive advanced NSCLC previously treated with crizotinib

Basel, May 19, 2017 - Novartis today announced the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has recommended approval of expanding the use of Zykadia® (ceritinib) to include the first-line treatment of patients with advanced non-small cell lung cancer (NSCLC) whose tumors are anaplastic lymphoma kinase (ALK)-positive. If approved, Zykadia will provide a new treatment option for previously untreated and newly diagnosed patients with ALK-positive advanced NSCLC.

"Novartis is committed to bringing targeted treatment options to more patients living with lung cancer who may benefit from them," said Bruno Strigini, CEO, Novartis Oncology. "Today, we've taken an important step towards fulfilling that commitment with the potential approval of Zykadia as a first-line treatment option for those in the EU diagnosed with ALK-positive advanced NSCLC."

The positive CHMP opinion was based on results from the ASCEND-4 study, a randomized, open-label, global Phase III trial. The study showed that patients treated with first-line Zykadia experienced a 45% reduction in the risk of disease progression compared to patients treated with standard first-line pemetrexed-platinum chemotherapy with pemetrexed maintenance (hazard ratio [HR] = 0.55 [95% CI: 0.42, 0.73]). The median progression-free survival (PFS) was 16.6 months (95% confidence interval [CI]: 12.6, 27.2) for patients receiving Zykadia compared to 8.1 months (95% CI: 5.8, 11.1) for patients in the chemotherapy arm of the study.

Additionally, patients receiving Zykadia without brain metastases at baseline experienced a median PFS of 26.3 months (95% CI: 15.4, 27.7), compared with 8.3 months (95% CI: 6.0, 13.7) among patients treated in the chemotherapy arm (HR = 0.48 [95% CI: 0.33, 0.69]). Among patients with brain metastases at baseline, the median PFS was 10.7 months (95% CI: 8.1, 16.4) in the Zykadia group versus 6.7 months (95% CI: 4.1, 10.6) in the chemotherapy group (HR = 0.70 [95% CI: 0.44, 1.12]). Of these patients, 59% did not receive prior brain radiotherapy. The high intracranial overall response rate (ORR) (72.7% [95% CI: 49.8, 89.3]) was consistent with whole body ORR (72.5% [95% CI: 65.5, 78.7]).

The CHMP recommendation will now be reviewed by the European Commission (EC), which holds the authority to approve medicines for the European Union (EU). The EC typically follows the CHMP recommendation and typically issues an approval decision within two months, applicable to all 28 European Union member states plus Iceland, Lichtenstein, and Norway. Earlier this year, the US Food and Drug Administration (FDA) granted Zykadia Breakthrough Therapy designation for first-line treatment of patients with ALK-positive NSCLC with metastases to the brain. The application for first-line use of Zykadia is under Priority Review by the FDA.

Novartis Commitment to Lung Cancer

Worldwide, lung cancer causes more deaths than colon, breast and prostate cancer combined, and an estimated 1.8 million new cases of lung cancer are diagnosed each year. Among patients with NSCLC, roughly 25% have an actionable mutation that may be targeted with available therapies. To determine that treatment, medical organizations recommend biomarker testing for patients with lung cancer.

Over the past decade, Novartis Oncology's research has supported the evolution of treatment approaches for patients living with mutation-driven types of lung cancer. The company continues its commitment to the global lung cancer community through ongoing studies, as well as the exploration of investigational compounds that target genomic biomarkers in NSCLC.

About ASCEND-4

ASCEND-4 was a Phase III randomized, open-label, multicenter, global clinical trial to evaluate the safety and efficacy of Zykadia compared to standard chemotherapy, including maintenance, in adult patients with Stage IIIB or IV ALK-positive advanced NSCLC who received no prior therapy for their advanced disease. Patients received Zykadia orally at 750 mg/daily or standard pemetrexed-based platinum doublet chemotherapy (pemetrexed 500 mg/m2 plus cisplatin 75 mg/m2 or carboplatin AUC 5-6) for four cycles followed by pemetrexed maintenance.

Of 376 patients, 189 (59 with brain metastases) were randomized to Zykadia and 187 (62 with brain metastases) to chemotherapy. Approximately 60% of patients with baseline brain metastases treated with Zykadia did not have prior radiation therapy, the current standard of treatment for baseline brain metastases.

The most common adverse events (AEs) occurring in more than 25% of Zykadia patients were diarrhea (85% vs. 11% with chemotherapy), nausea (69% vs. 55% with chemotherapy), vomiting (66% vs. 36% with chemotherapy), ALT increase (60% vs. 22% with chemotherapy), AST increase (53% vs. 19% with chemotherapy), GGT increase (37% vs. 10% in chemotherapy), decreased appetite (34% vs. 31% with chemotherapy), blood alkaline phosphate increase (29% vs. 5% with chemotherapy) and fatigue (29% vs. 30% with chemotherapy).

About Zykadia

Zykadia is an oral, selective inhibitor of anaplastic lymphoma kinase (ALK), a gene that can fuse with others to form an abnormal "fusion protein" that promotes the development and growth of certain tumors in cancers including non-small cell lung cancer (NSCLC). Zykadia is currently

approved in over 69 countries worldwide. Please visit www.NovartisOncology.com/news/product-portfolio/zykadia (link is external) for additional information.

Zykadia Important Safety Information
Zykadia may cause serious side effects.

Zykadia may cause stomach upset and intestinal problems in most patients, including diarrhea, nausea, vomiting and stomach-area pain. These problems can be severe. Patients should follow their doctor's instructions about taking medicines to help these symptoms, and should call their doctor for advice if symptoms are severe or do not go away.

Zykadia may cause severe liver injury. Patients should have blood tests prior to the start of treatment with Zykadia, every two weeks for the first three months of treatment and monthly thereafter, and should talk to their doctor right away if they experience any of the following symptoms: tiredness (fatigue), itchy skin, yellowing of the skin or the whites of the eyes, nausea or vomiting, decreased appetite, pain on the right side of the abdomen, urine turns dark or brown, or bleeding or bruising more easily than normal.

Zykadia may cause severe or life-threatening swelling (inflammation) of the lungs during treatment that can lead to death. Symptoms may be similar to those symptoms from lung cancer. Patients should tell their doctor right away about any new or worsening symptoms, including trouble breathing or shortness of breath, fever, cough, with or without mucous, or chest pain.

Zykadia may cause very slow, very fast, or abnormal heartbeats. Doctors should check their patient's heart during treatment with Zykadia. Patients should tell their doctor right away if they feel new chest pain or discomfort, dizziness or lightheadedness, faint, or have abnormal heartbeats, blue discoloration of lips, shortness of breath, swelling of lower limbs or skin, or if they start to take or have any changes in heart or blood pressure medicines.

Zykadia may cause high levels of glucose in the blood. People who have diabetes or glucose intolerance, or who take a corticosteroid medicine have an increased risk of high blood sugar with Zykadia. Patients should have glucose blood tests prior to the start of treatment with Zykadia and during treatment. Patients should follow their doctor's instructions about blood sugar monitoring and call their doctor right away with any symptoms of high blood sugar, including increased thirst and/or urinating often.

Zykadia may cause high levels of pancreatic enzymes in the blood and may cause pancreatitis. Patients should have blood tests prior to the start of treatment with Zykadia and as needed during their treatment with Zykadia. Patients should talk to their doctor if they experience signs and symptoms of pancreatitis which including upper abdominal pain that may spread to the back and get worse with eating.

Before patients take Zykadia, they should tell their doctor about all medical conditions, including liver problems; diabetes or high blood sugar; heart problems, including a condition called long QT

syndrome; if they are pregnant, if they think they may be pregnant, or if they plan to become pregnant; are breastfeeding or plan to breastfeed.

Zykadia may harm unborn babies. Women who are able to become pregnant must use a highly effective method of birth control (contraception) during treatment with Zykadia and up to 3 months after stopping Zykadia. It is not known if Zykadia passes into breast milk. Patients and their doctor should decide whether to take Zykadia or breastfeed, but should not do both.

Patients should tell their doctor about medicines they take, including prescription medicines, over-the-counter medicines, vitamins and herbal supplements. If they take Zykadia while using oral contraceptives, the oral contraceptives may become ineffective.

The most common adverse reactions with an incidence of >=10% were diarrhea, nausea, vomiting, liver laboratory test abnormalities (requires blood test monitoring), tiredness (fatigue), abdominal pain, decreased appetite, weight decreased, constipation, kidney laboratory test abnormalities (requires blood test monitoring), rash, anemia and heartburn. Grade 3-4 adverse reactions with an incidence of >=5% were liver laboratory test abnormalities, tiredness (fatigue), vomiting, hyperglycemia (requires blood test monitoring), nausea and diarrhea.

Patients should stop taking Zykadia and seek medical help immediately if they experience any of the following, which may be signs of an allergic reaction:

- Difficulty in breathing or swallowing
- Swelling of the face, lips, tongue or throat
- Severe itching of the skin, with a red rash or raised bumps

Patients should tell their doctor of any side effect that bothers them or does not go away. These are not all of the possible side effects of Zykadia. For more information, patients should ask their doctor or pharmacist.

Patients should take Zykadia exactly as their health care provider tells them. Patients should not change their dose or stop taking Zykadia unless their health care provider advises them to. Zykadia should be taken once a day on an empty stomach. Patients should not eat for at least 2 hours before and 1 hour after taking Zykadia. If a dose of Zykadia is missed, they should take it as soon as they remember. If their next dose is due within the next 12 hours, they should skip the missed dose and take the next dose at their regular time. They should not take a double dose to make up for a forgotten dose. Patients should not drink grapefruit juice or eat grapefruit during treatment with Zykadia, as it may make the amount of Zykadia in their blood increase to a harmful level. If patients have to vomit after swallowing Zykadia capsules, they should not take more capsules until their next scheduled dose.

Disclaimer
The foregoing release contains forward-looking statements that can be identified by words such as "positive CHMP opinion," "recommended," "will," "committed," "may," "step towards," "commitment," "potential," "recommendation," "Breakthrough Therapy designation," "Priority

Review," "ongoing," "investigational," or similar terms, or by express or implied discussions regarding potential new indications or labeling for Zykadia, or regarding potential future revenues from Zykadia. You should not place undue reliance on these statements. Such forward-looking statements are based on the current beliefs and expectations of management regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that Zykadia will be submitted or approved for any additional indications or labeling in any market, or at any particular time. Nor can there be any guarantee that Zykadia will be commercially successful in the future. In particular, management's expectations regarding Zykadia could be affected by, among other things, the uncertainties inherent in research and development, including clinical trial results and additional analysis of existing clinical data; regulatory actions or delays or government regulation generally; the company's ability to obtain or maintain proprietary intellectual property protection; general economic and industry conditions; global trends toward health care cost containment, including ongoing pricing and reimbursement pressures; safety, quality or manufacturing issues, and other risks and factors referred to in Novartis AG's current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this press release as of this date and does not undertake any obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.